

CDER New Drug Review: 2016 Update

John K. Jenkins, M.D.
Director
Office of New Drugs
Center for Drug Evaluation and Research

FDA/CMS Summit
December 14, 2016

Housekeeping

- Data and analyses presented on the following slides are thought to be accurate. In order to provide the most up-to-date information the analyses have not undergone the same thorough quality control as is performed for official FDA reports
- Many staff in CDER provided data, analyses, and PowerPoint expertise for this talk; their work behind the scenes makes me look good each year. Special thanks and acknowledgement to:
 - The Performance Analysis and Data Services Staff in CDER's Office of Program and Strategic Analysis
 - Mike Lanthier in the Office of the Commissioner
- Pay attention to fiscal year (**FY**) or calendar year (**CY**) and **cut-off dates** on data presentations

Topics to be covered

- How is CDER doing with regard to meeting PDUFA goals?
- What are the trends in new drug development and approvals?
 - IND activity, NME submissions, and NME approvals
 - Utilization and impact of expedited programs
- Update on Breakthrough Therapy Designation Program

CDER PDUFA Review Performance



	FY 2015			FY 2016	
Submission Type	Number Filed	Performance (Current)		Number Filed	Performance (Potential)**
Priority NME NDAs/original BLAs	24	92%		20	100%
Standard NME NDAs/original BLAs	22	100%		17	100%
Priority non-NME NDAs/BLAs*	9	100%		11	91%
Standard non-NME NDAs/BLAs*	84	95%		74	99%
Class 1 NDA/BLA Resubmissions	6	100%		5	100%
Class 2 NDA/BLA Resubmissions	35	97%		31	100%
Priority Efficacy Supplements	52	94%		45	100%
Standard Efficacy Supplements	123	94%		110	97%
Class 1 Efficacy Resubmissions	0	--		2	100%
Class 2 Efficacy Resubmissions	10	60%		10	90%
Prior Approval Mfg Supplements	544	91%		560	96%
CBE Mfg Supplements	1237	95%		1109	99%

Data as of 9/30/2016

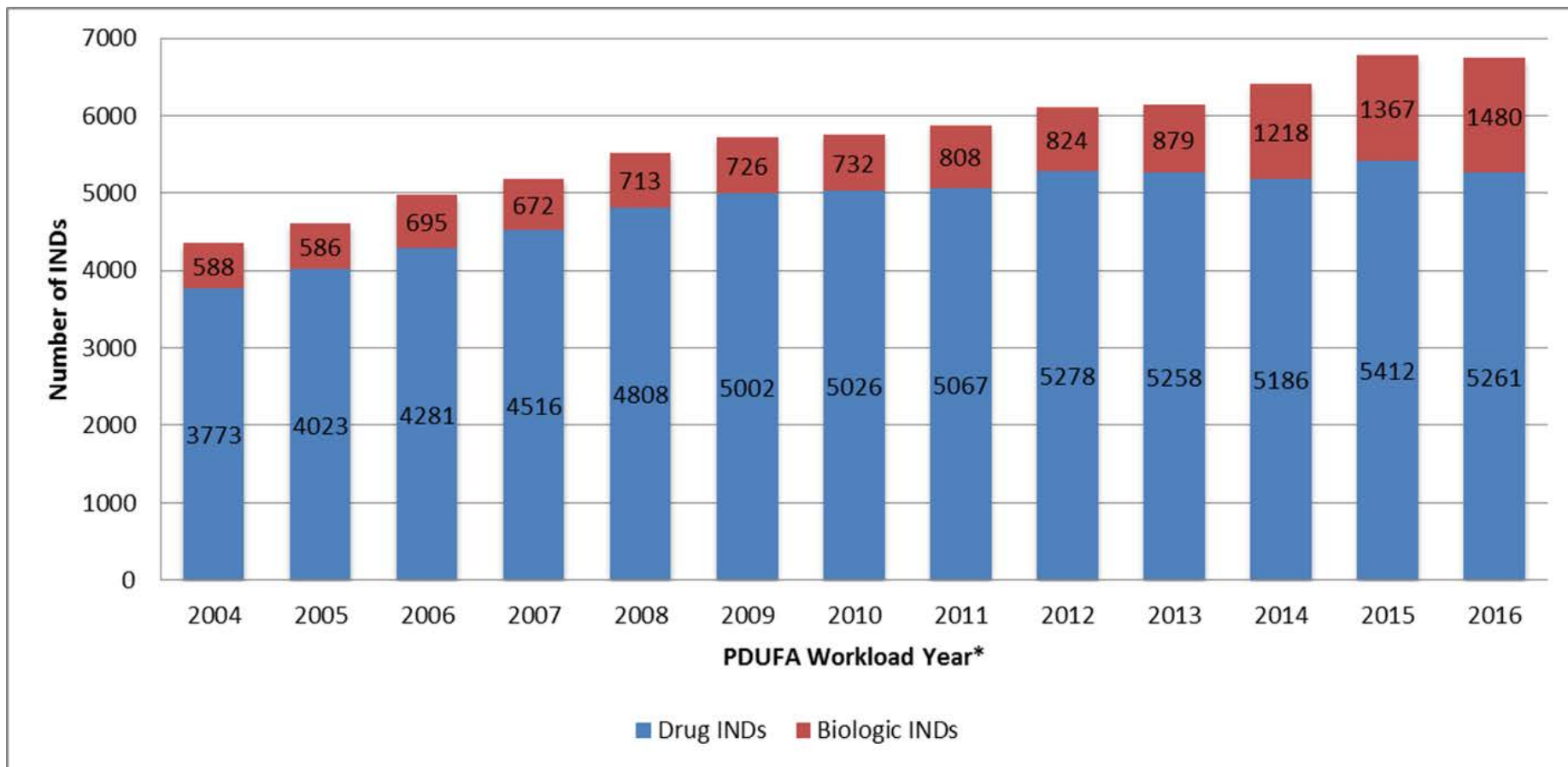
*Beginning in FY 2013, the new tracked metrics are non-NME Priority and non-NME Standard NDAs.

† Includes submissions pending filing.

**Potential Performance refers to the level of performance that could potentially be achieved if all the actions currently pending are reviewed within their required goal date.

Submissions with unknown review schedules are excluded.

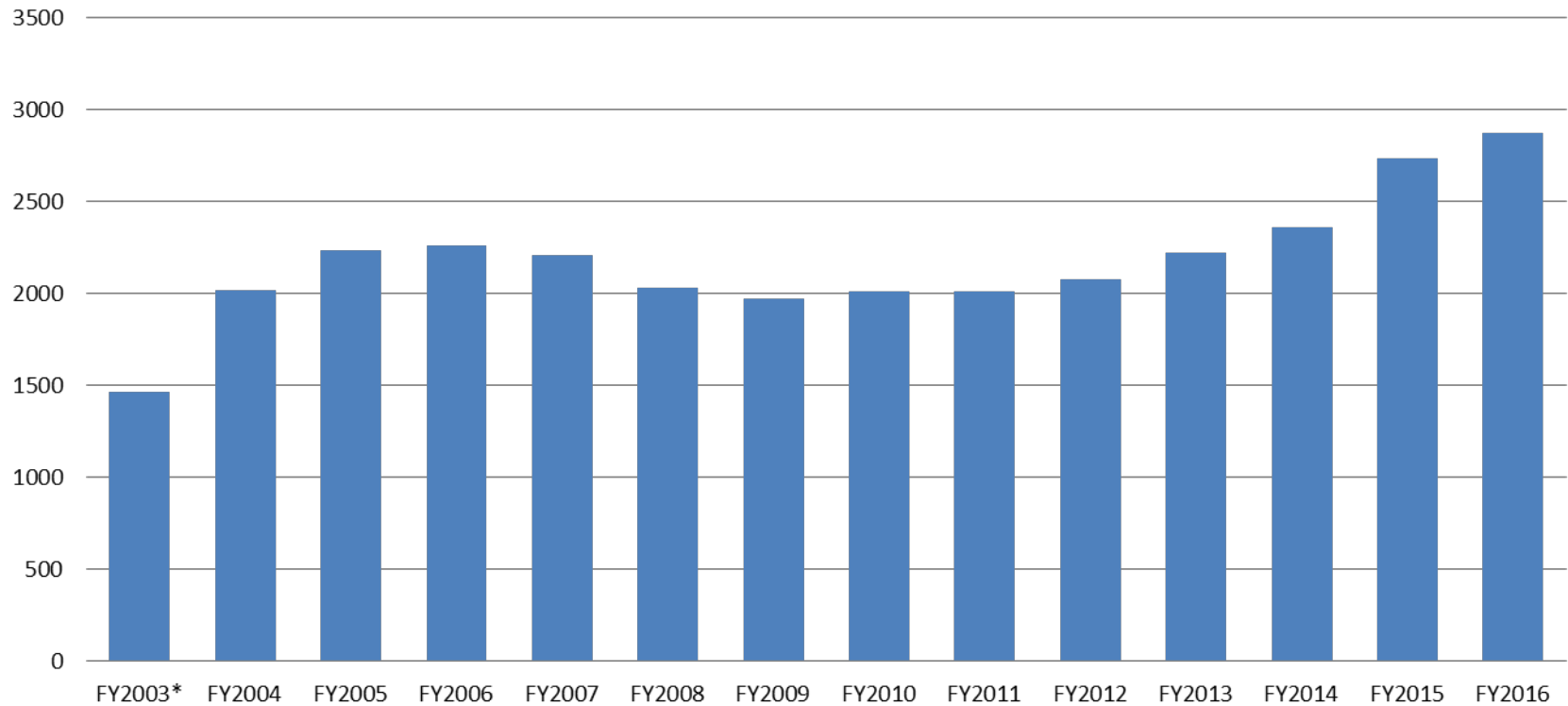
Commercial INDs With Activity Based On PDUFA Workload Adjuster Data



Data represent 12 month period of July 1st - June 30th

CDER PDUFA

Formal Meeting Requests



*BLAs were not included as they transferred to CDER in FY2004

Data as of 9/30/2016

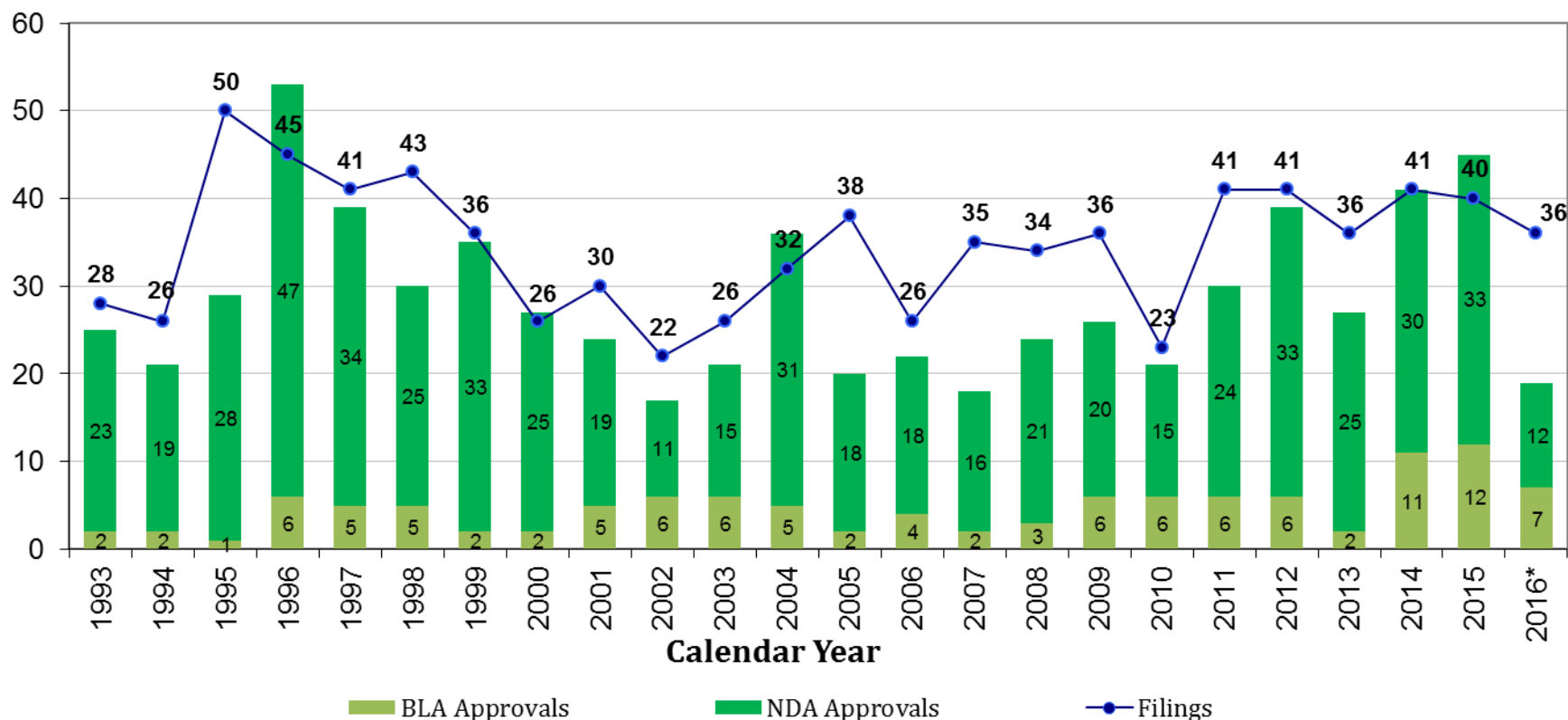
What About Novel New Drug Approvals?

- For CY16, through **December 9th, 2016**, CDER has:
 - Received **36** NME applications; average NME filings for past decade is 35
 - Approved **19** NMEs*, including **7** Orphan Drugs
- Reasons for fewer NMEs compared to CY15?
 - Approval of 5 NMEs in CY15 with CY16 due dates
 - Fewer NME actions in CY16
 - Increased number of CR letters in CY16

* This information is accurate as of December 9th, 2016. In rare instances, it may be necessary for FDA to change a drug's new molecular entity (NME) designation or the status of its application as a novel new biologics license application (BLA). For instance, new information may become available which could lead to a reconsideration of the original designation or status. If changes must be made to a drug's designation or the status of an application as a novel BLA, the Agency intends to communicate the nature of, and the reason for, any revisions as appropriate. This note applies to all references to NME/Original BLAs in this presentation.

CDER NME NDAs/BLAs[†]

Filings and Approvals as of 12/9/16

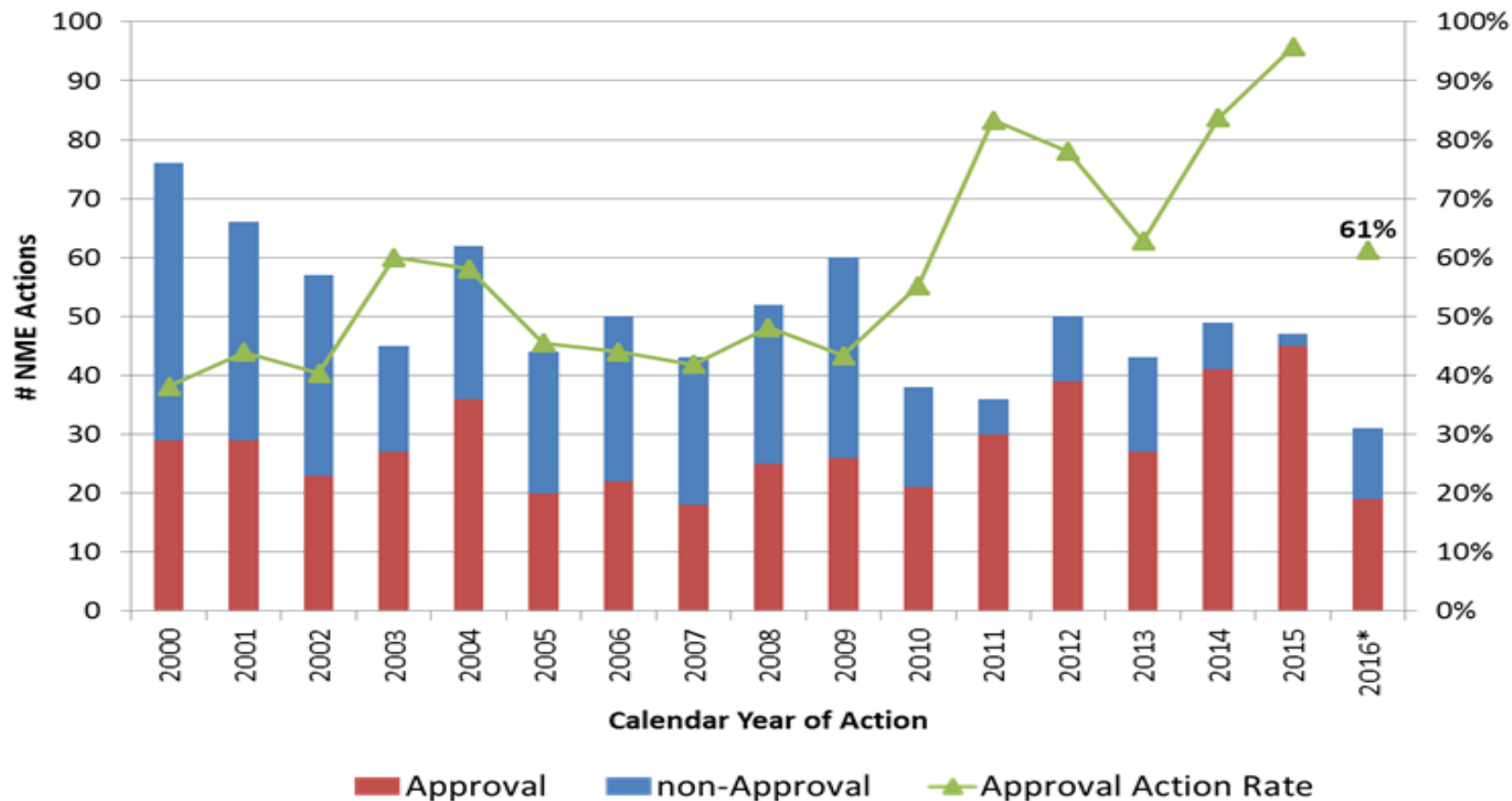


[†] Multiple applications pertaining to a single new molecular/biologic entity are only counted once. Original BLAs that do not contain a new active ingredient are excluded.

This information is accurate as of December 9th, 2016. In rare instances, it may be necessary for FDA to change a drug's new molecular entity (NME) designation or the status of its application as a novel new biologics license application (BLA). For instance, new information may become available which could lead to a reconsideration of the original designation or status. If changes must be made to a drug's designation or the status of an application as a novel BLA, the Agency intends to communicate the nature of, and the reason for, any revisions as appropriate. This note applies to all references to NME/Original BLAs in this presentation.

*Since applications are received and filed throughout a calendar year, the filed applications in a given calendar year do not necessarily correspond to an approval in the same calendar year. Certain applications are within their 60-day filing review period and may not be filed upon completion of the review.

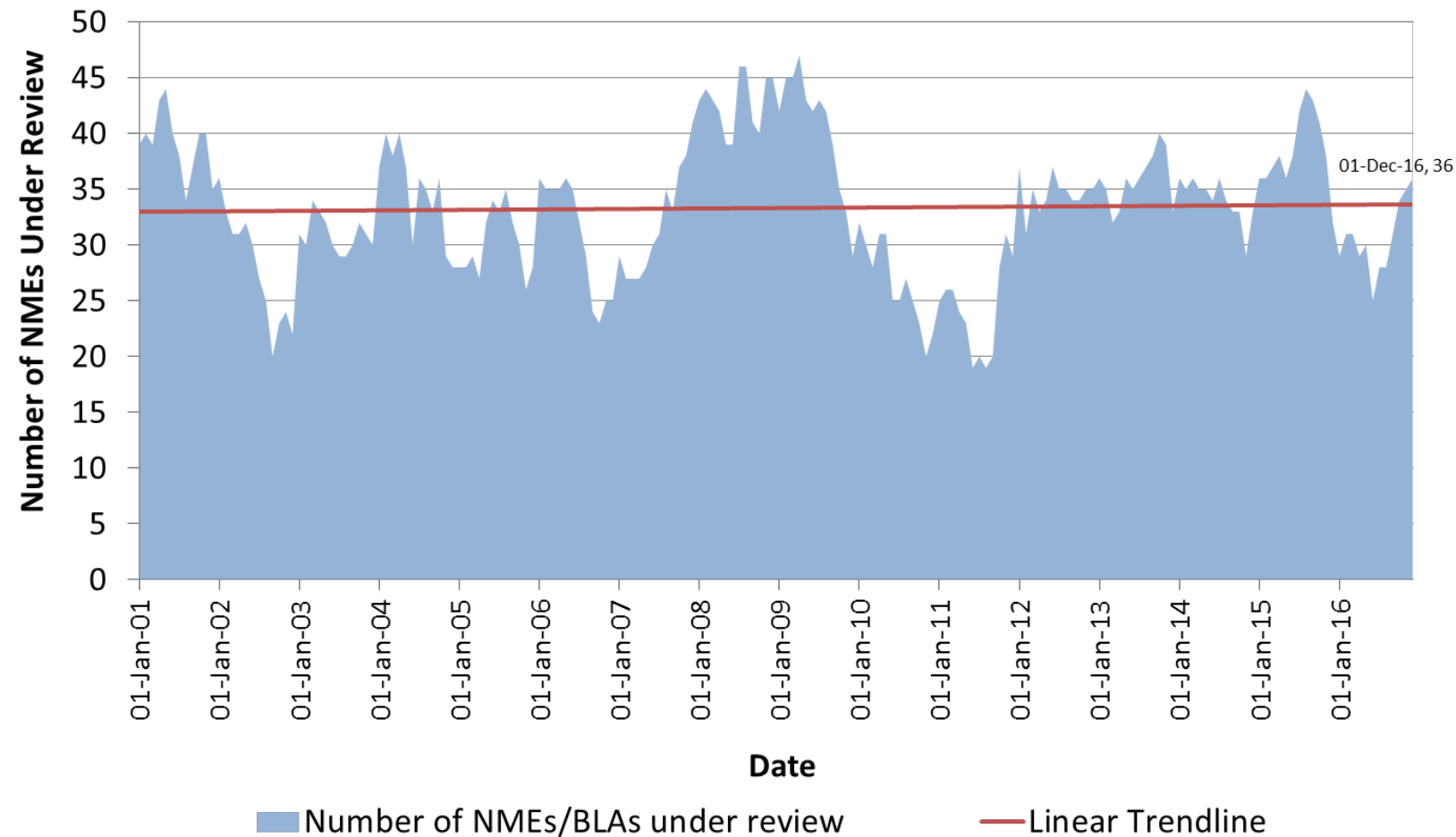
NME Actions and Approvals



*Data as of 12/9/2016

Includes discrete actions on a given date for an active ingredient which, if approved, would constitute a new molecular entity. Actions for original submissions and resubmissions as well as actions for new BLAs are included. Multiple actions which occur on the same date for multiple dosage forms or indications are counted as a single regulatory action.

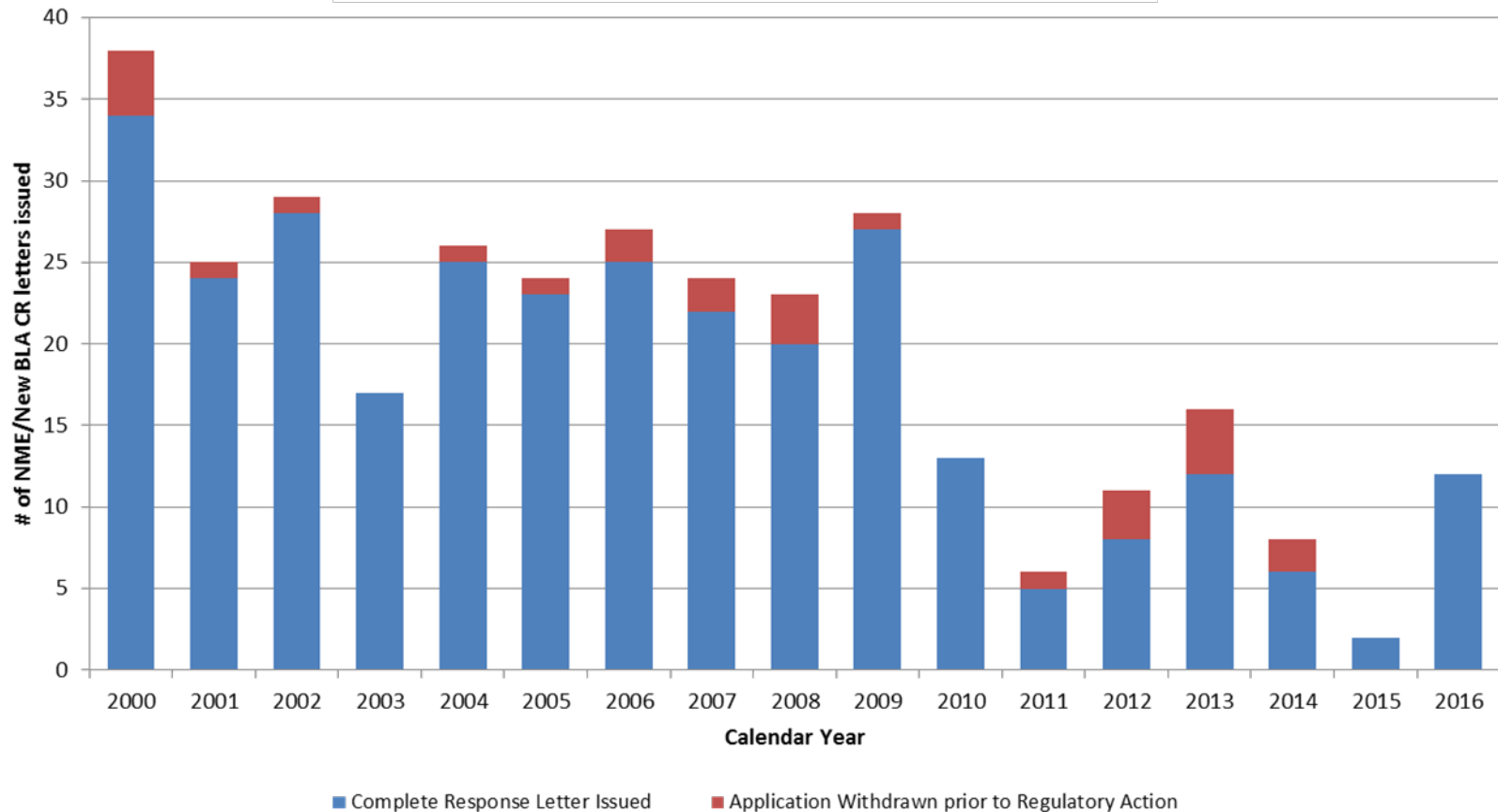
CDER Novel Drugs and Biologics Under Active Review



* Data as of 12/1/2016

CDER NME/New BLA

Complete Response* Letters Issued

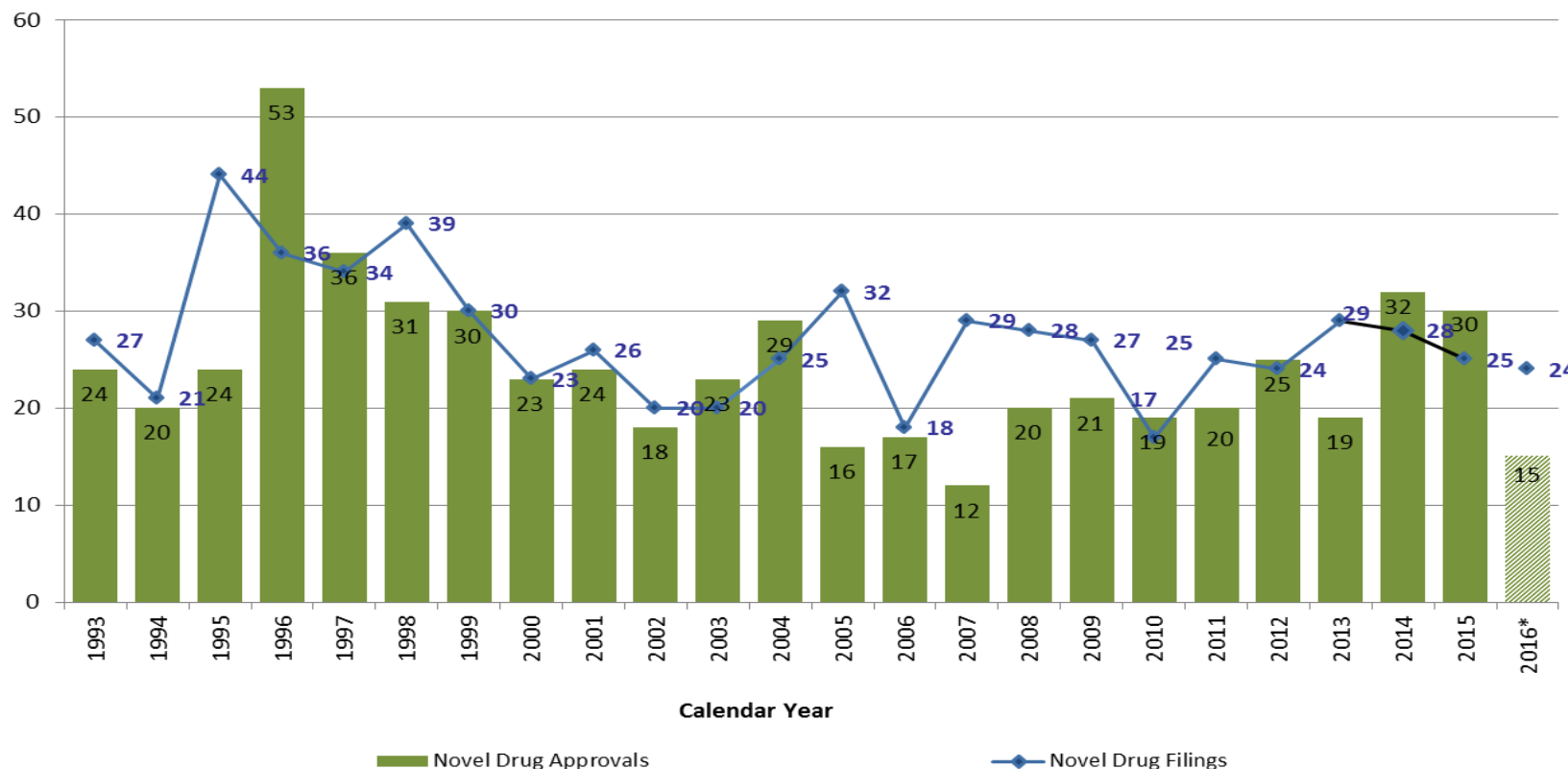


Data as of 12/9/2016

* Complete Response letter figures include “approvable” and “not approvable” letters issued for NDA actions prior to August 11, 2008, the date the Complete Response Letter rule was finalized.

CDER NME NDAs/BLAs[†]

Filings and Approvals for non-OHOP Products



[†] Multiple applications pertaining to a single new molecular/biologic entity are only counted once. Original BLAs that do not contain a new active ingredient are excluded.

This information is accurate as of December 9th, 2016. In rare instances, it may be necessary for FDA to change a drug's new molecular entity (NME) designation or the status of its application as a novel new biologics license application (BLA). For instance, new information may become available which could lead to a reconsideration of the original designation or status. If changes must be made to a drug's designation or the status of an application as a novel BLA, the Agency intends to communicate the nature of, and the reason for, any revisions as appropriate. This note applies to all references to NME/Original BLAs in this presentation.

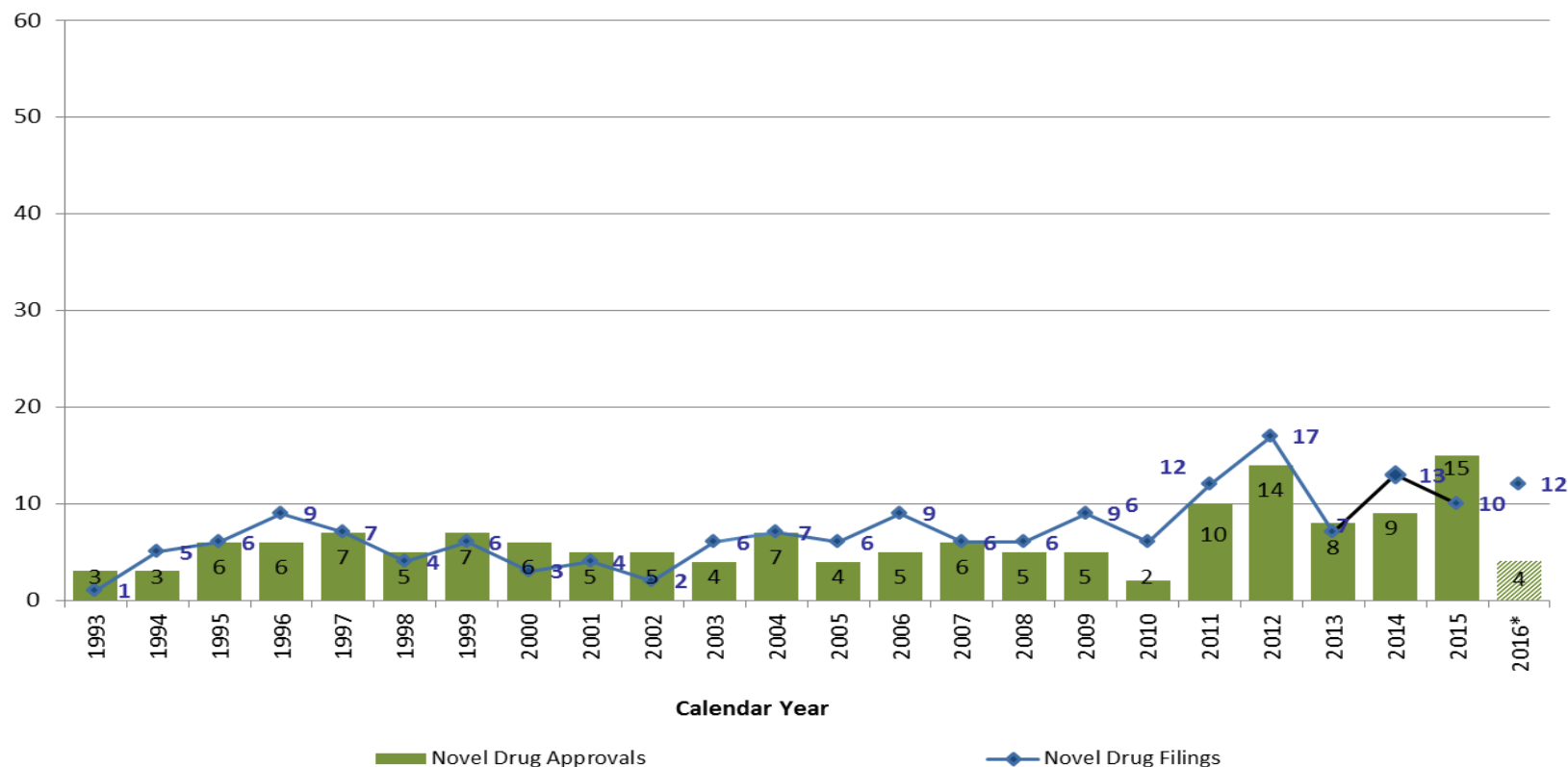
*Since applications are received and filed throughout a calendar year, the filed applications in a given calendar year do not necessarily correspond to an approval in the same calendar year. Certain applications are within their 60-day filing review period and may not be filed upon completion of the review.

* Data through 12/9/2016

CDER NME NDAs/BLAs[†]



Filings and Approvals for OHOP Products



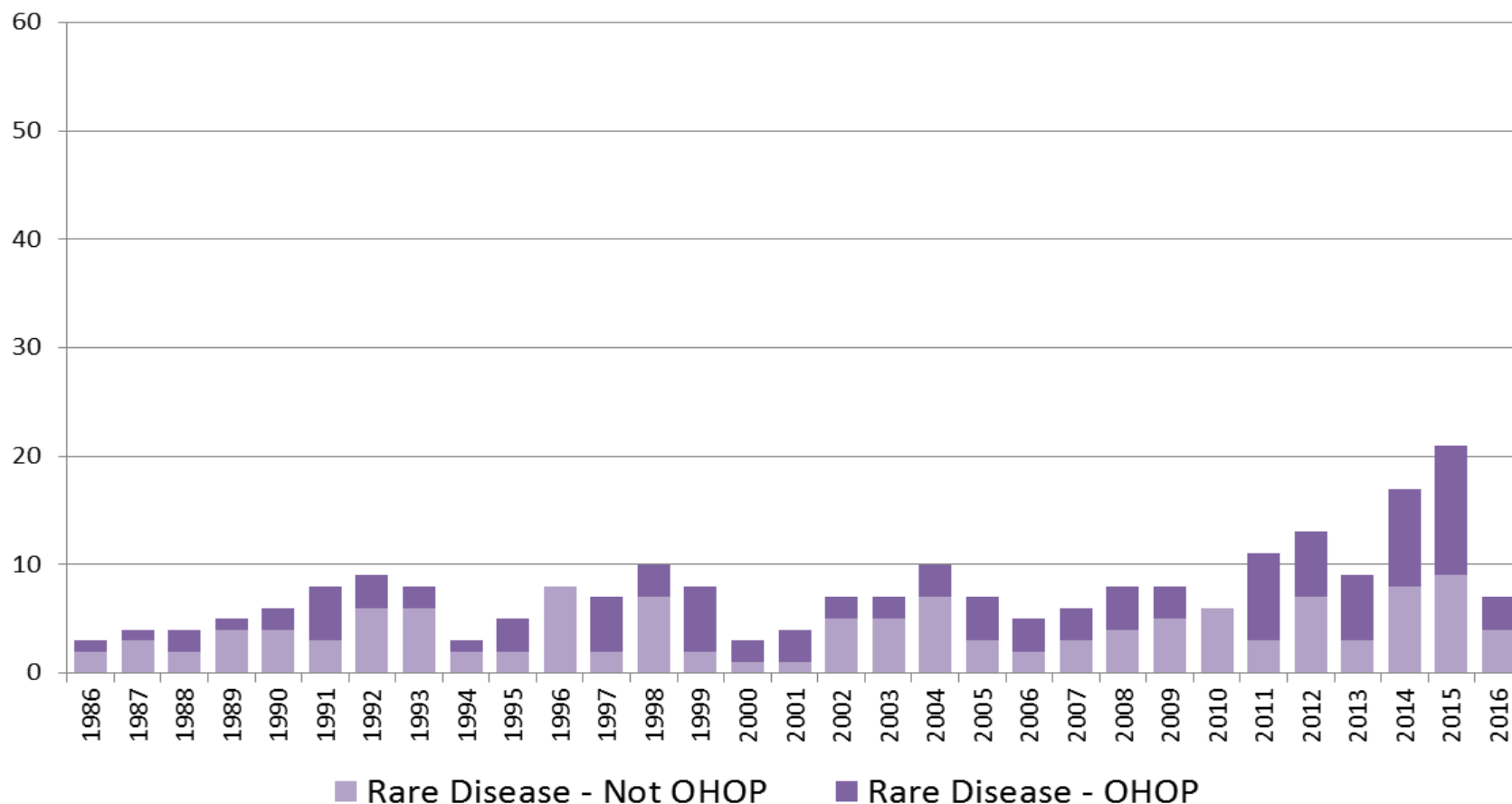
[†] Multiple applications pertaining to a single new molecular/biologic entity are only counted once. Original BLAs that do not contain a new active ingredient are excluded.

This information is accurate as of December 9th, 2016. In rare instances, it may be necessary for FDA to change a drug's new molecular entity (NME) designation or the status of its application as a novel new biologics license application (BLA). For instance, new information may become available which could lead to a reconsideration of the original designation or status. If changes must be made to a drug's designation or the status of an application as a novel BLA, the Agency intends to communicate the nature of, and the reason for, any revisions as appropriate. This note applies to all references to NME/Original BLAs in this presentation.

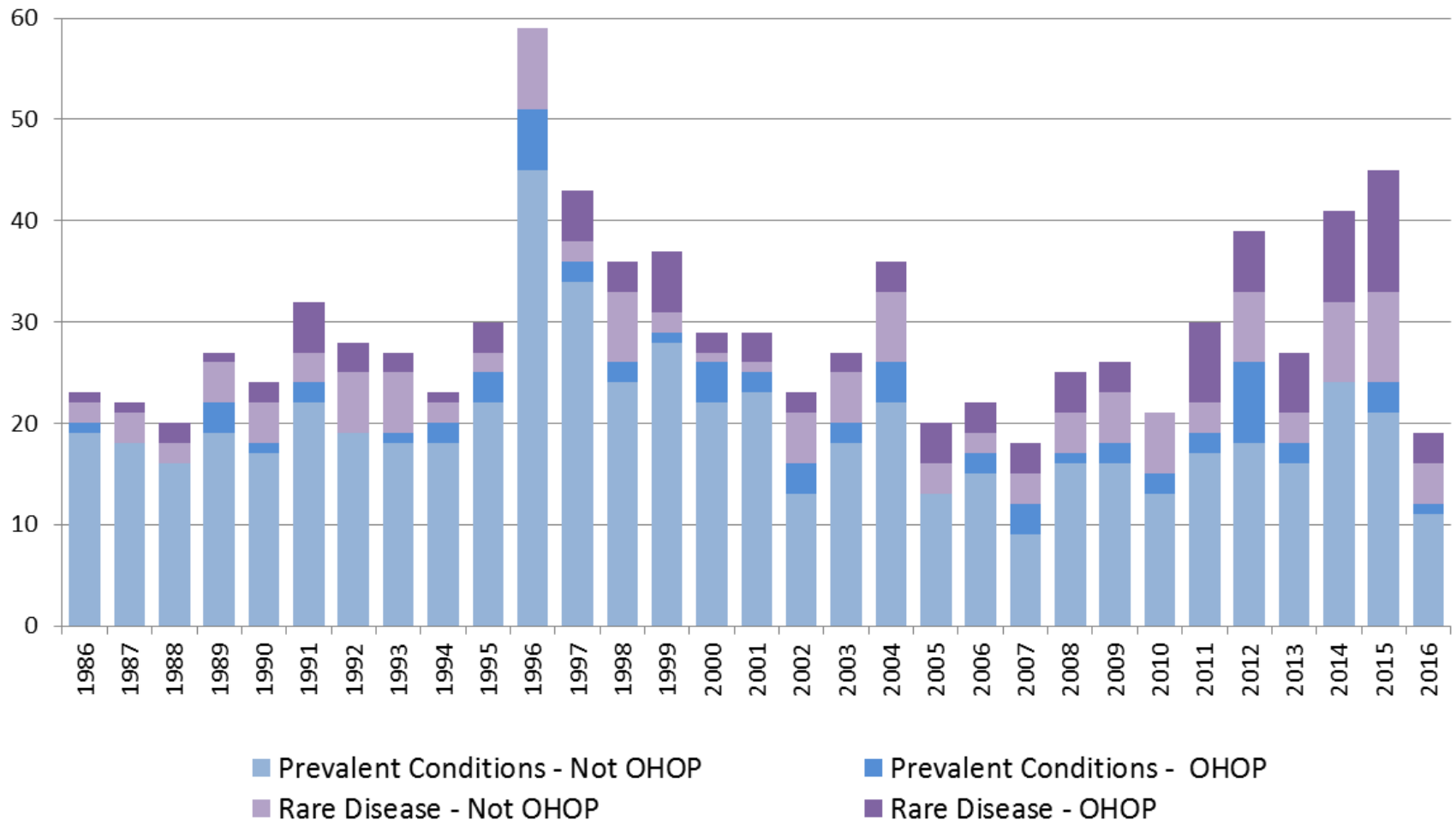
*Since applications are received and filed throughout a calendar year, the filed applications in a given calendar year do not necessarily correspond to an approval in the same calendar year. Certain applications are within their 60-day filing review period and may not be filed upon completion of the review.

* Data through 12/9/2016

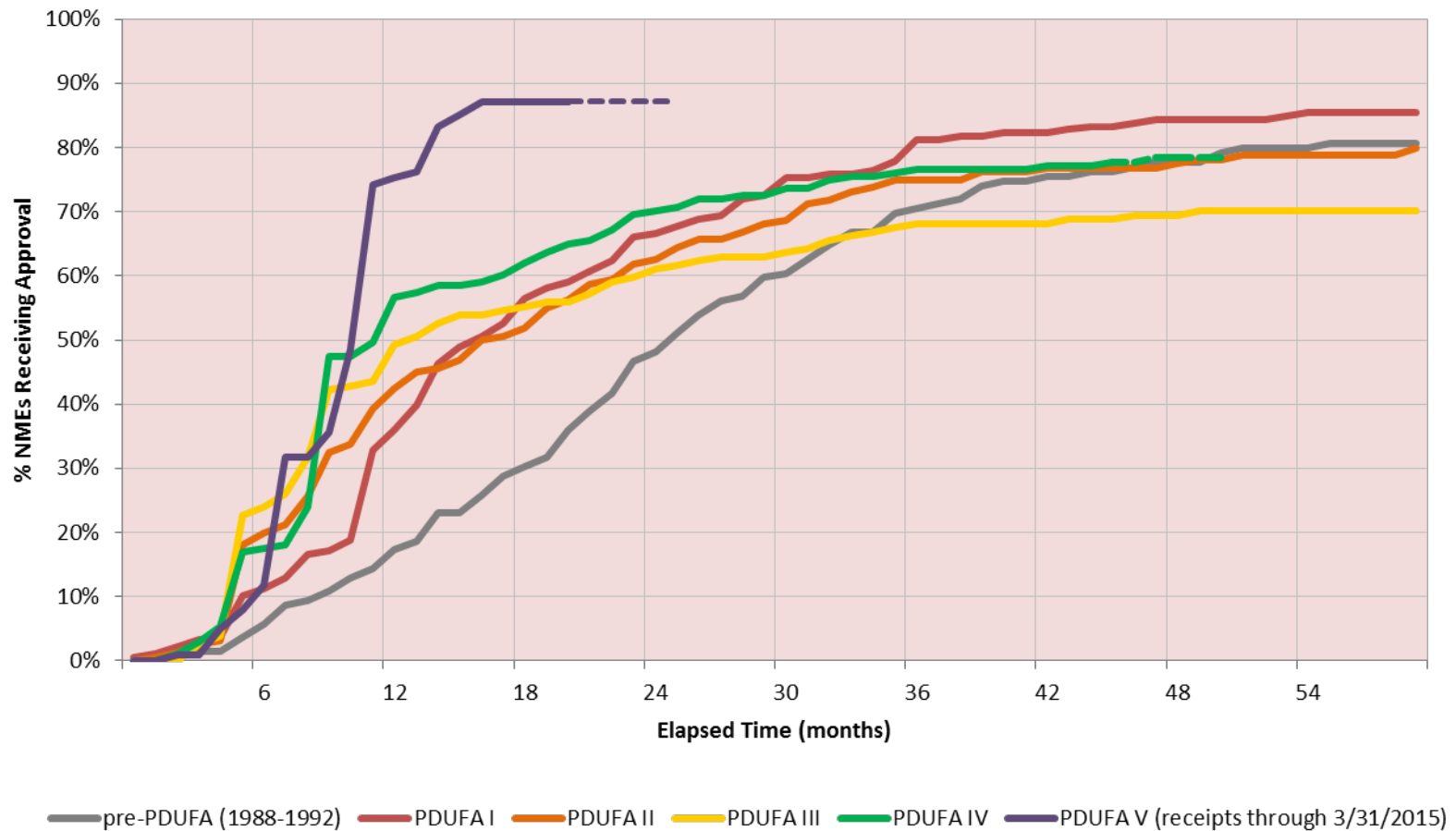
Novel Rare Disease Approvals



Novel Drug Approvals by OHOP/non-OHOP and Disease Prevalence



CDER New Molecular Entity Approval Rates by PDUFA Cohort



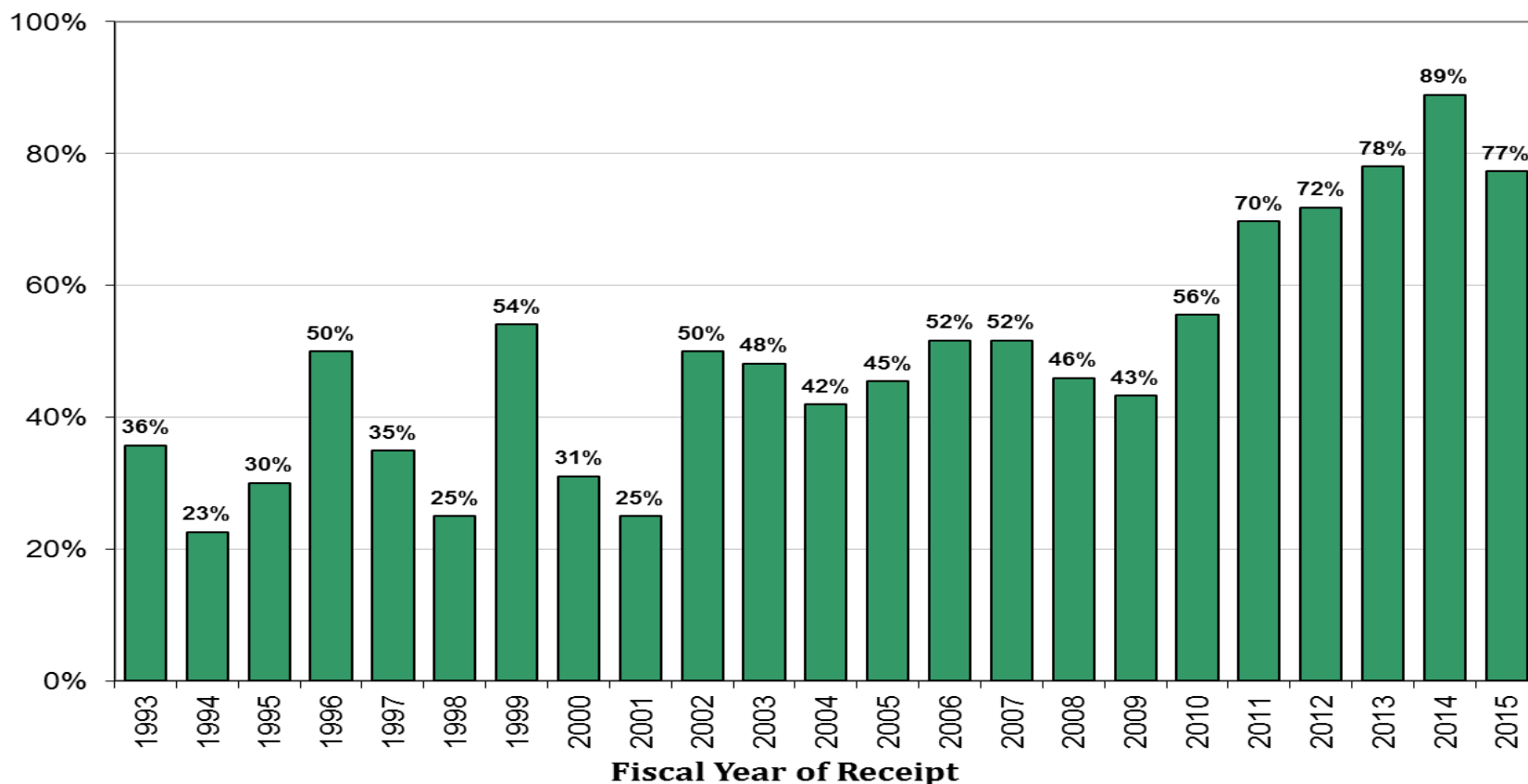
* PDUFA V estimates based on 77 NMEs submitted in FY 2013 – mid FY 2015 (it is too early to estimate performance for later submissions)

Projection estimates account for actions to date and elapsed time to date for non-approvals

Data as of 9/30/16

CDER NME NDAs/BLAs[†]

First Action Approval Rate



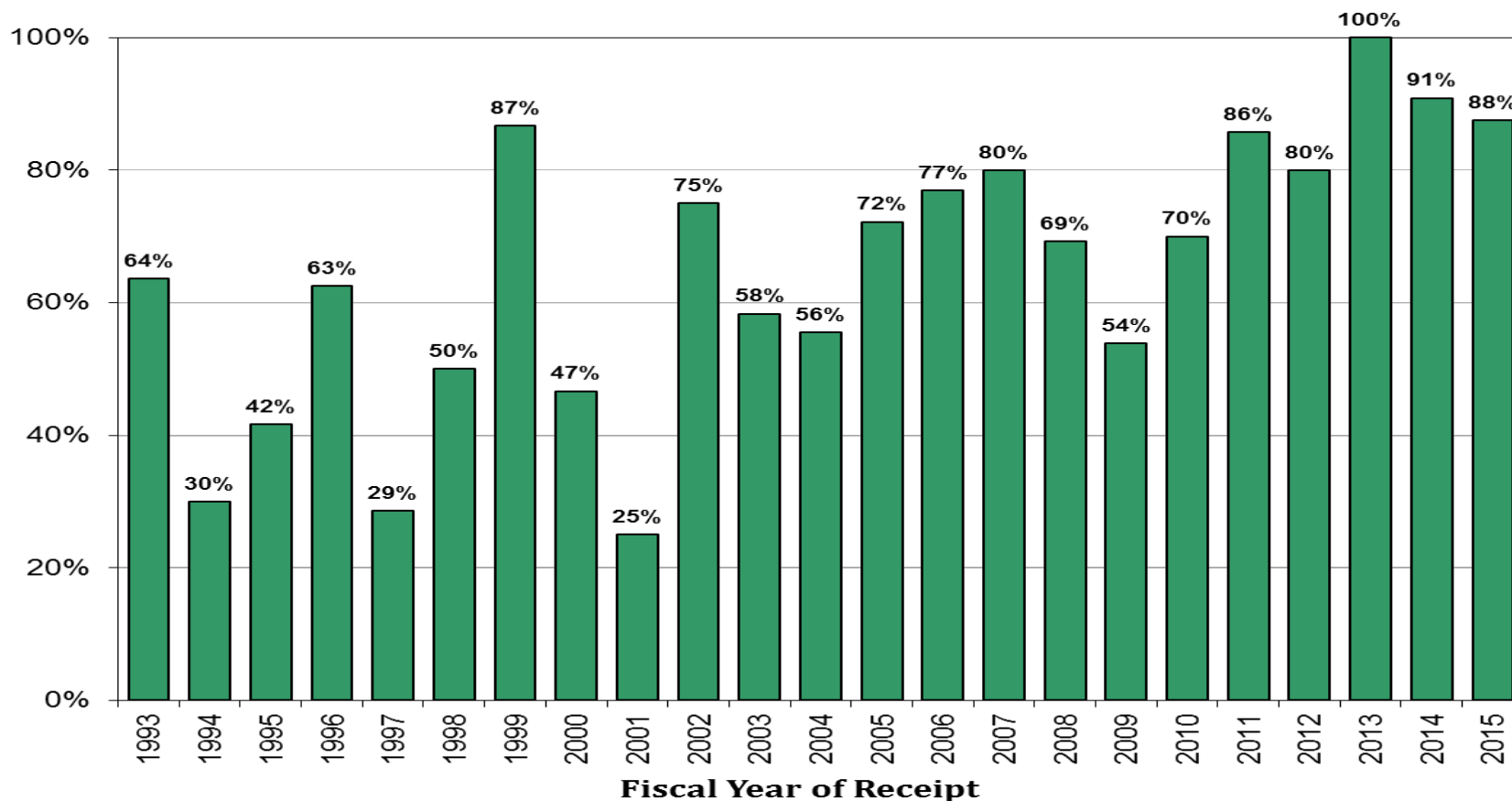
Data as of 12/9/2016

[†] Multiple applications pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers represented here for filings are not indicative of workload in the PDUFA V Program.

[†] Original BLAs that do not contain a new active ingredient are excluded.

Percentages exclude pending applications from the denominator.

CDER First Action Approval Rates For **Priority** NME NDAs/BLAs†



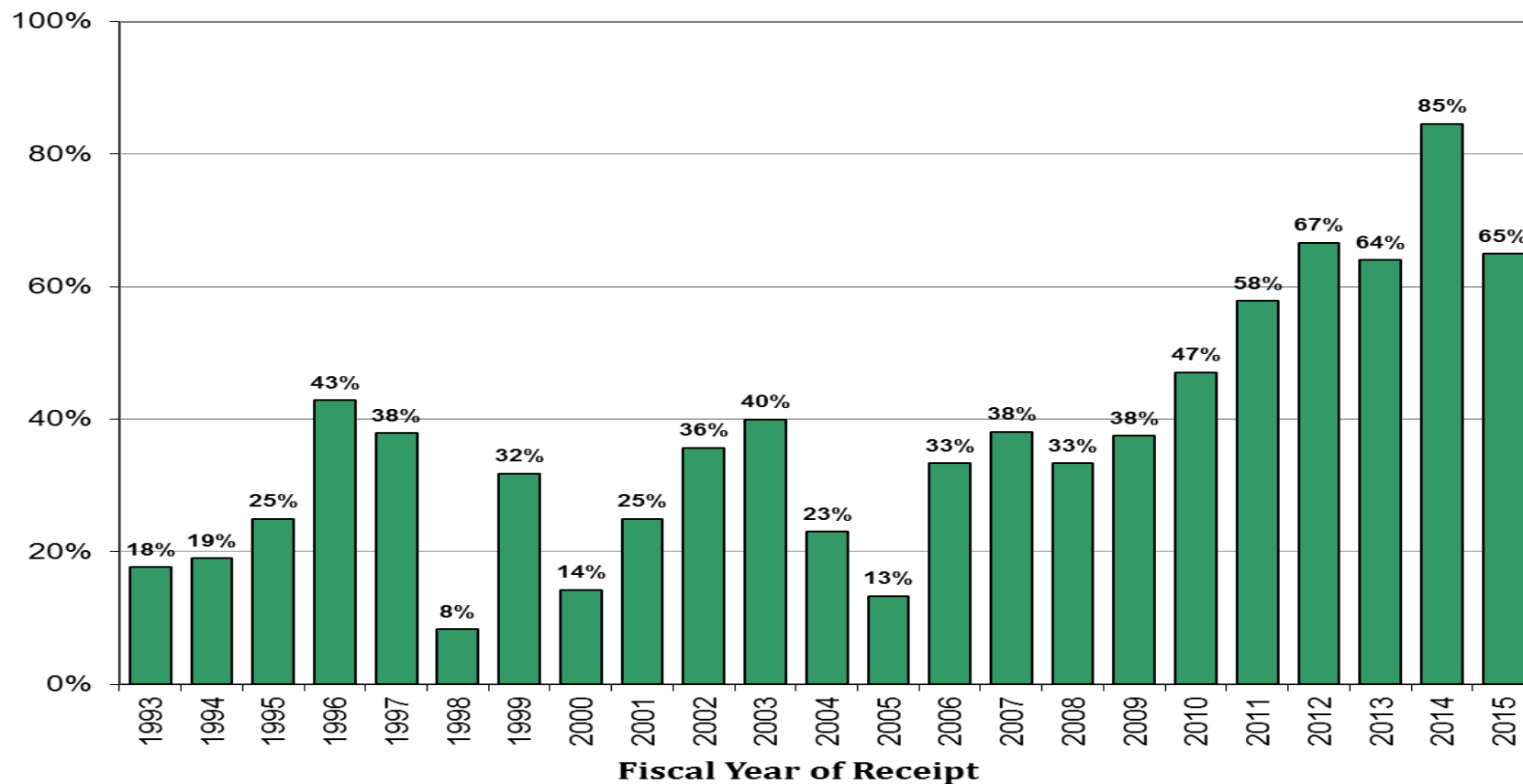
Data as of 12/9/2016

† Multiple submissions pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers represented here for filings are not indicative of workload in the PDUFA V Program.

† Original BLAs that do not contain a new active ingredient are excluded.

Percentages exclude pending applications from the denominator .

CDER First Action Approval Rates For **Standard** NME NDAs/BLAs[†]



Data as of 12/9/2016

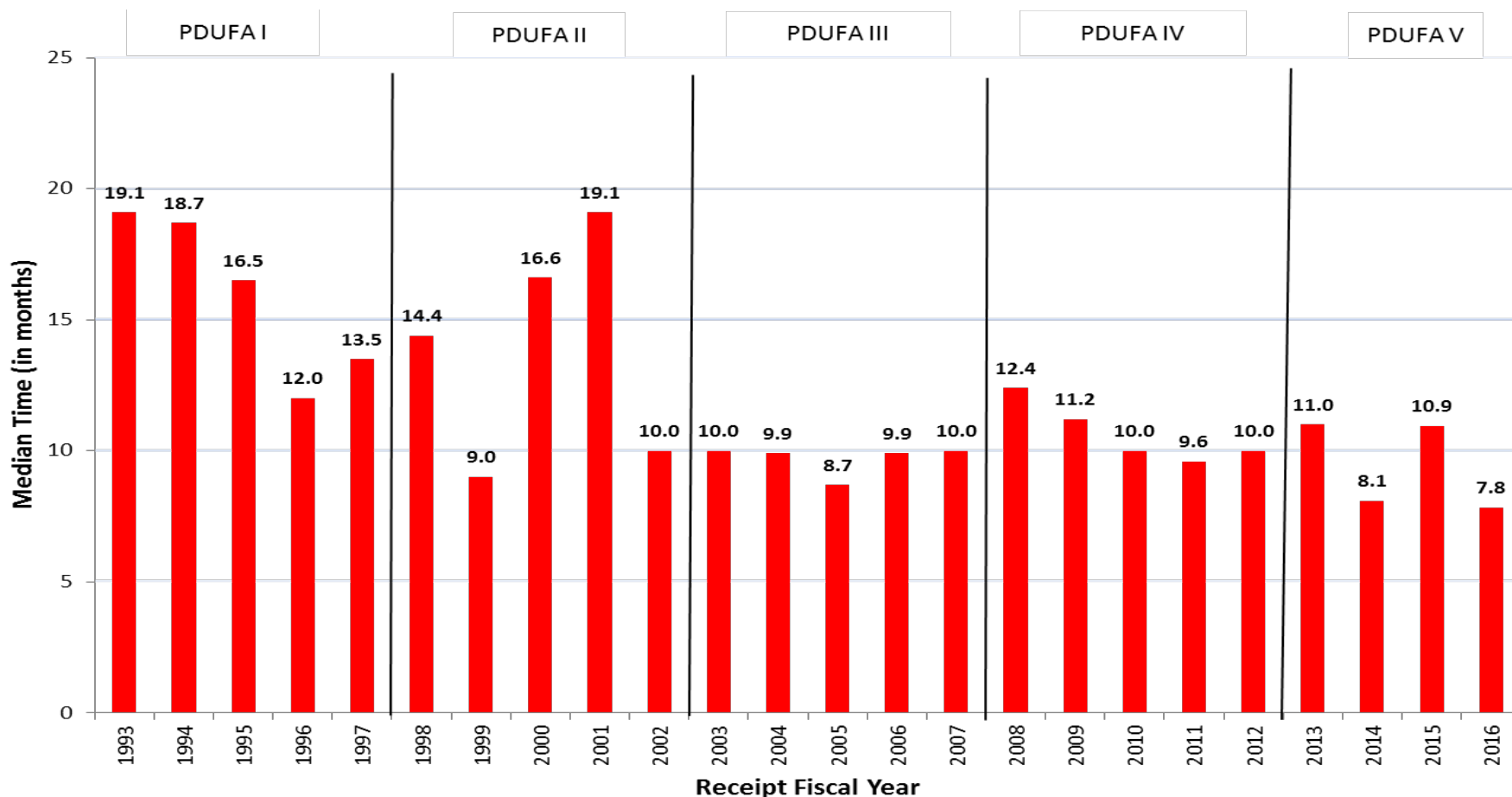
[†] Multiple submissions pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers represented here for filings are not indicative of workload in the PDUFA V Program.

[†] Original BLAs that do not contain a new active ingredient are excluded.

Percentages exclude pending applications from the denominator.

CDER Overall NME NDA/BLAs[†]

Median Total Time to Approval

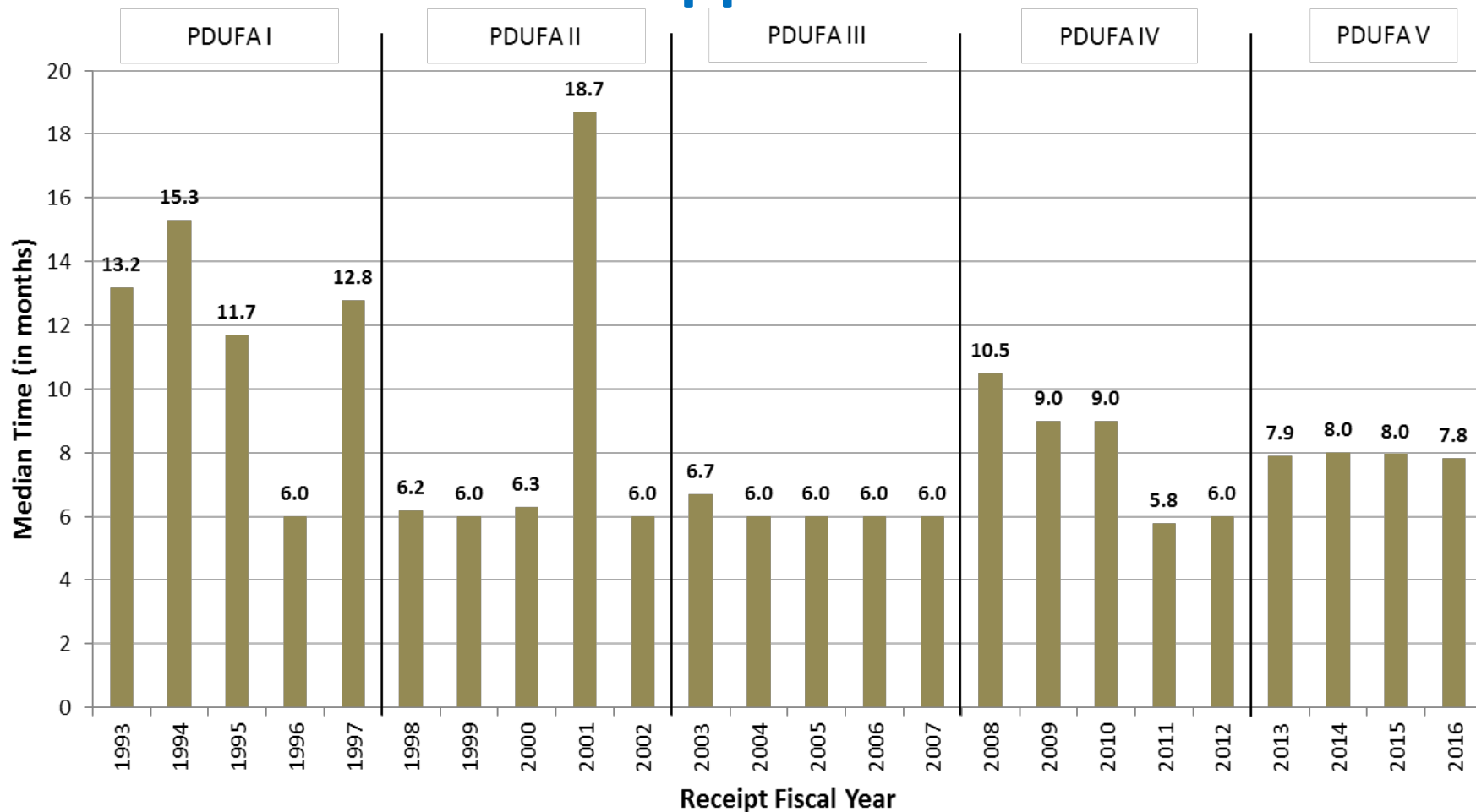


Data as of 12/9/2016

[†] Original BLAs that do not contain a new active ingredient are excluded.

CDER **Priority** NME NDAs/BLAs[†]

Median Total Time to Approval

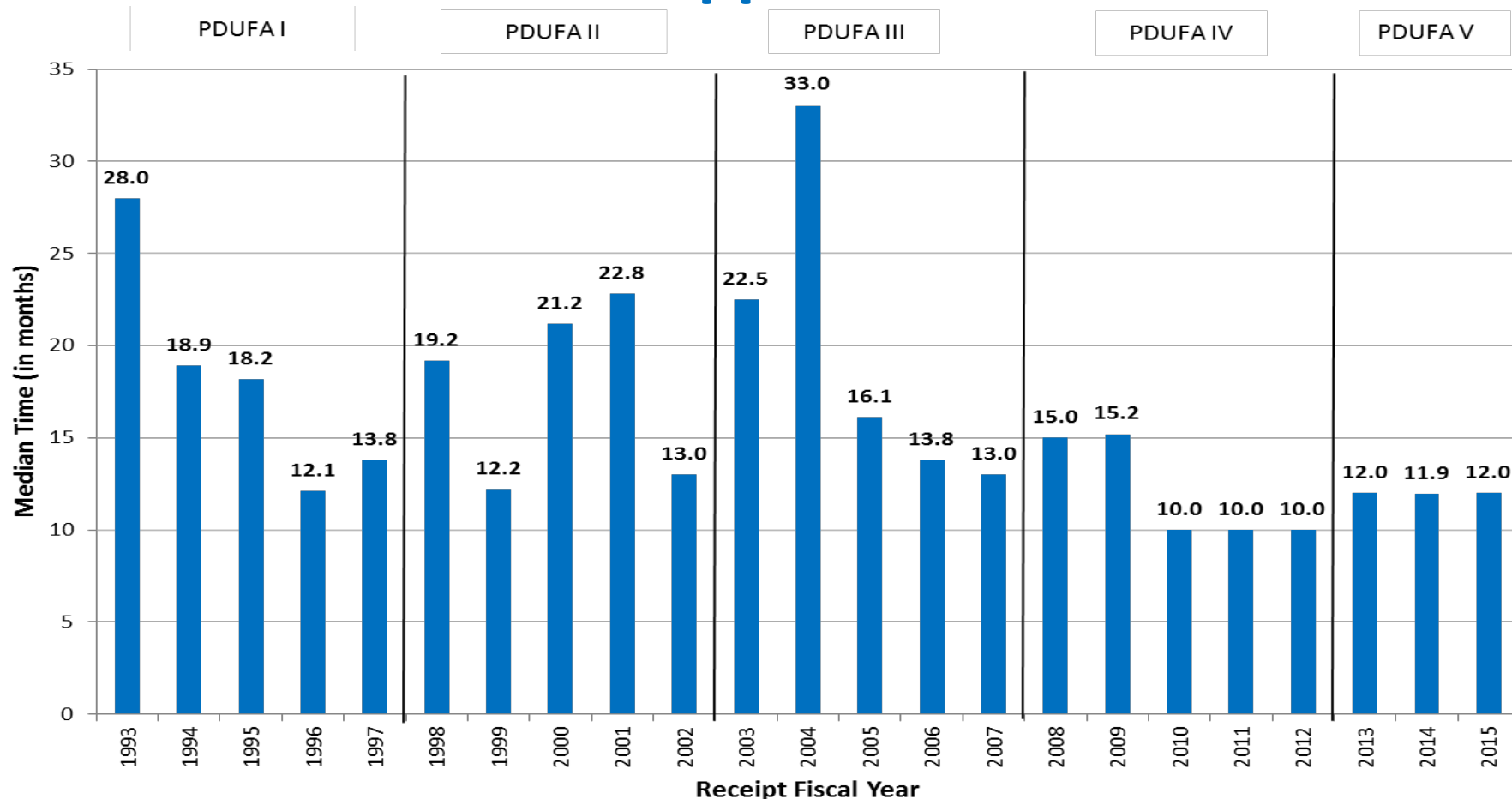


Data as of 12/9/2016

[†] Original BLAs that do not contain a new active ingredient are excluded.

CDER **Standard** NME NDA/BLAs[†]

Median Total Time to Approval

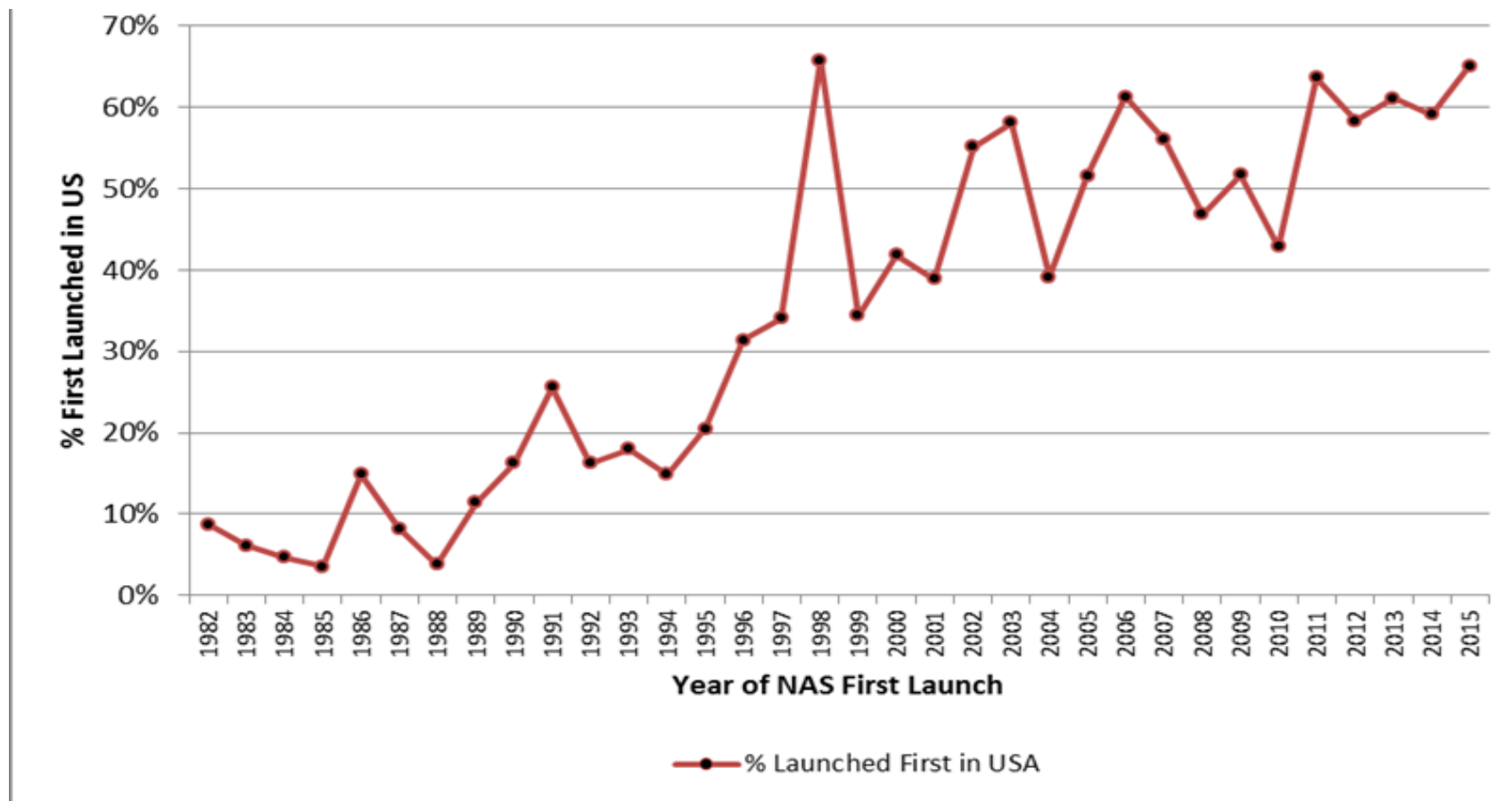


Data as of 12/9/2016

[†] Original BLAs that do not contain a new active ingredient are excluded.

No FY 16 Standard applications have been acted upon.

USA Share of New Active Substances Launched on World Market

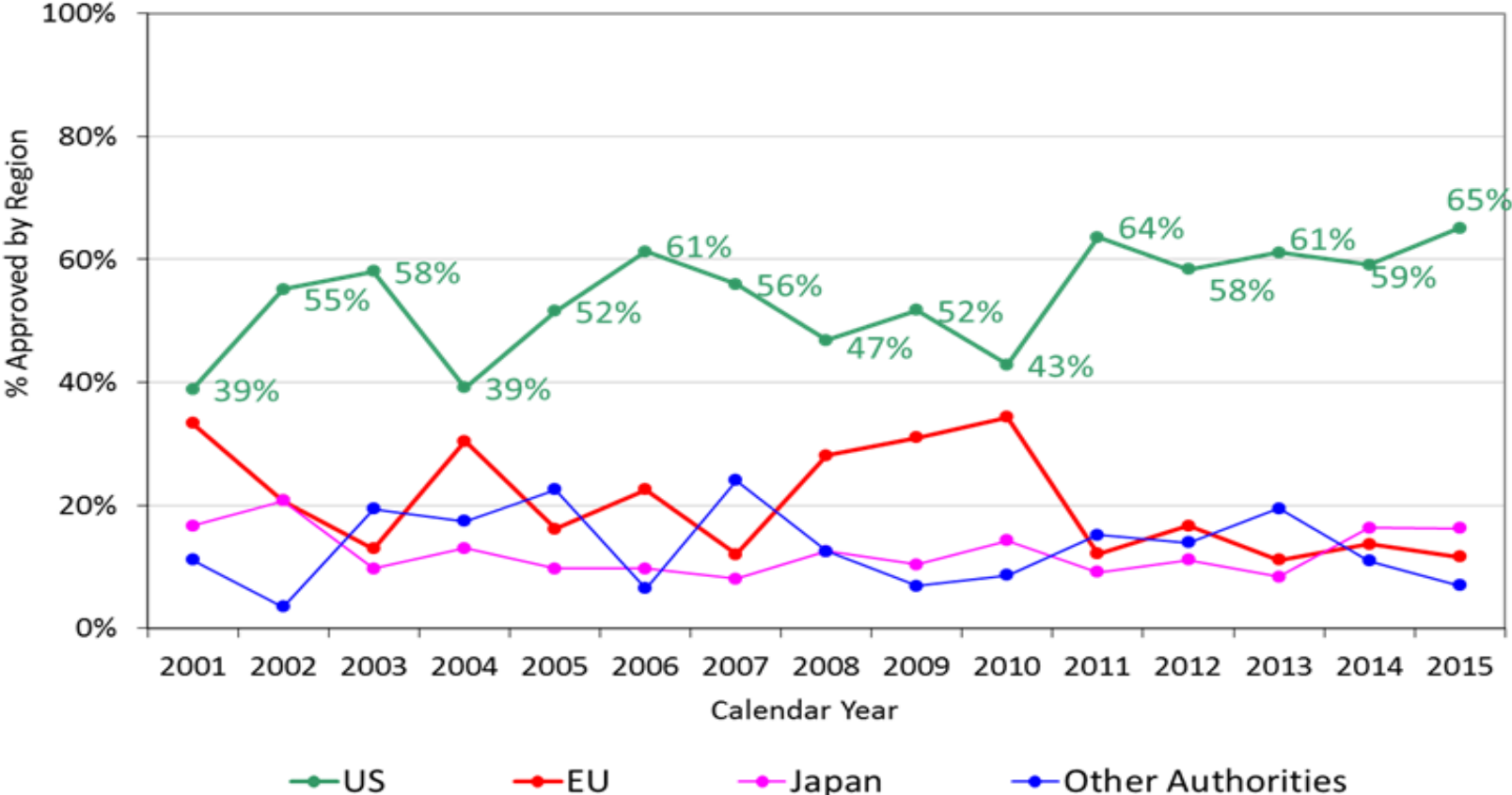


Data as of 11/30/2016

Source: *Scrip Magazine* (1982 - 2006), *Pharmaprojects/Citeline Pharma R&D Annual Review* (2007 - 2016)

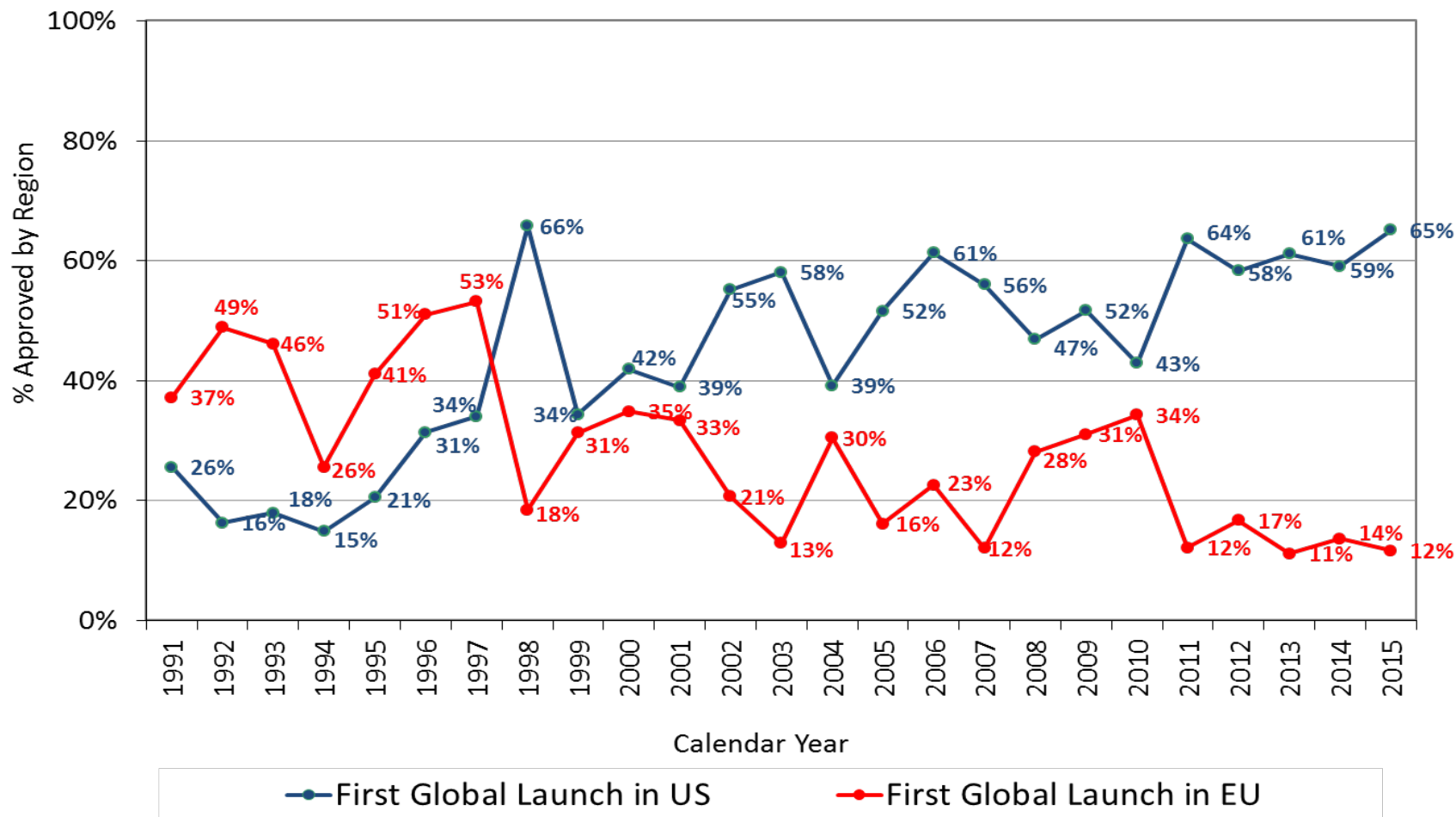
Global New Active Substances

First Launches by Region 2001 – 2015



Source: *Scrip Magazine* (2001 - 2006), *Pharmaprojects/Citeline Pharma R&D Annual Review* (2007 - 2016)

Global New Active Substance First Launches in the USA and EU: 1991 – 2015



Source: *Scrip Magazine* (1992 - 2006), *Pharmaprojects/Citeline Pharma R&D Annual Review* (2007 - 2016)

Snapshot of CY 2016

NME NDAs/BLAs[†] Drug Approvals



Trade Name	Met PDUFA Goal Date*	Approved on First Cycle	Priority Approval	Fast Track	First in Class	Approved First in the U.S.	Accelerated Approval	Orphan Drug	Breakthrough Therapy	QIDP
ZEPATIER										
BRIVIACT										
ANTHIM										
TALTZ										
CINQAIR										
DEFITELIO										
VENCLEXTA										
NUPLAZID										
TECENTRIQ										
ZINBRYTA										
OCALIVA										
AXUMIN										
NETSPOT										
EPCLUSA										
XIIDRA										
ADLYXIN										
EXONDYS 51										
LARTRUVO										
ZINPLAVA										

Data as of 12/9/2016

[†] Multiple submissions pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers are not indicative of workload in the PDUFA V Program.

[†] Original BLAs that do not contain a new active ingredient are excluded.

* A PDUFA Goal Date is marked as met if the NME is acted upon within its approval cycle due date.

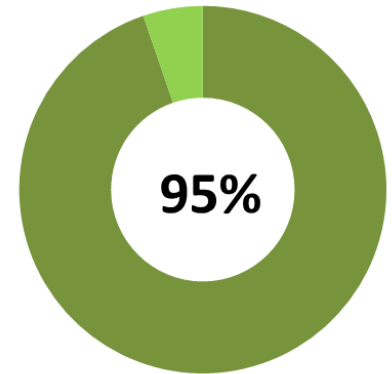
QIDP - Qualified Infectious Disease Product

In CY 2016, CDER Continued To Ensure The Efficiency Of First Cycle Review

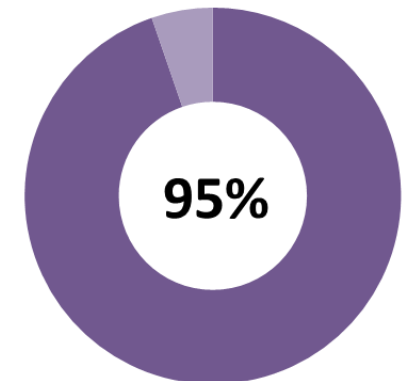


- All but one (95%) of the novel drugs approved to date in CY16 met their PDUFA goal dates for the approval review cycle
- Almost all (95%) of the novel drugs approved to date in CY16, were approved in the first review cycle

Met PDUFA Goal Date



Approved on First Cycle

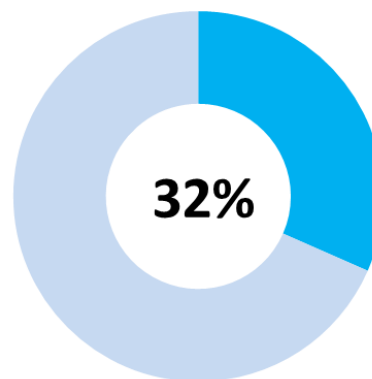


CDER Ensures That Novel Drugs Receive Expedited Review

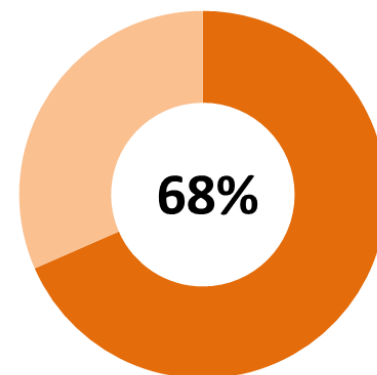


- About Two – thirds (68%) of the novel drugs approved to date in CY16 were approved under Priority Review
- Almost a third (32%) of the novel drugs approved to date in CY16 received Breakthrough Therapy designation
- About four out of ten (37%) of the novel drugs approved to date in CY16 received Fast Track designation

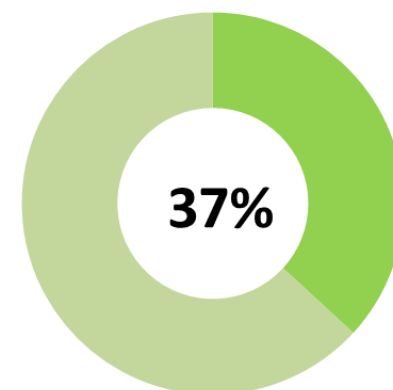
Breakthrough Therapy



Priority Approval



Fast Track

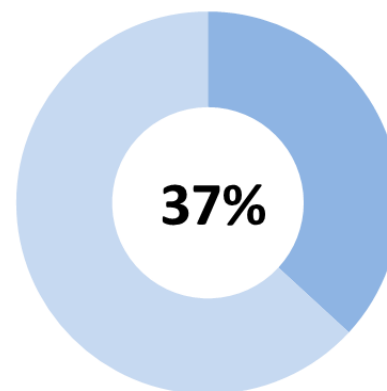


2016 Continues A Strong Track Record For Drug Innovation

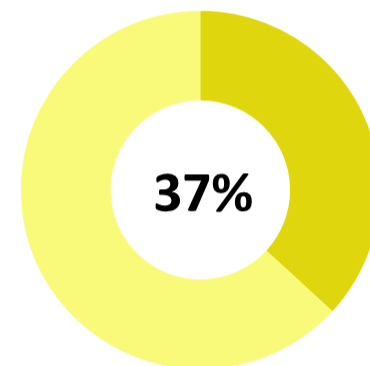


- Over a third (37%) of the novel drugs approved to date in CY16 are for rare diseases
- Almost four out of ten (37%) of the novel drugs approved to date in CY16 are the first in their class
- About eight out of ten (84%) of the novel drugs approved to date in CY16 were first approved in the U.S.

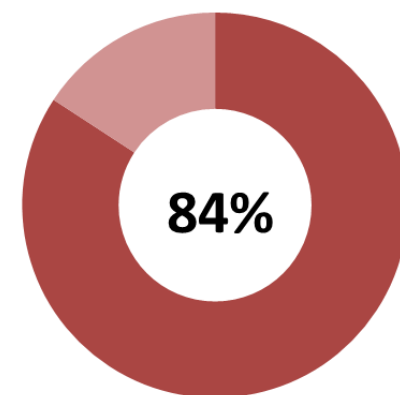
First in Class



Orphan Drugs



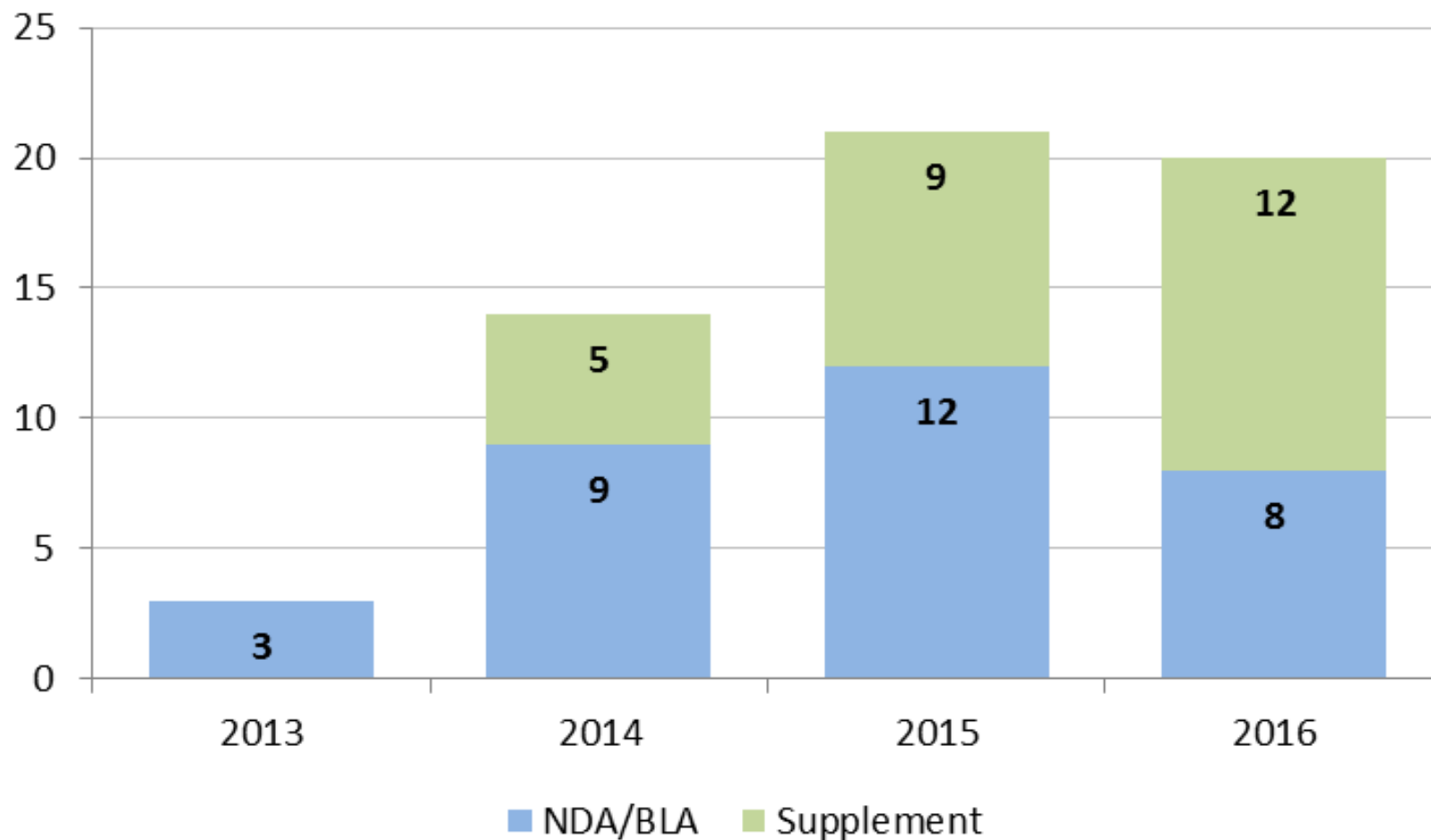
Approved First in the
United States



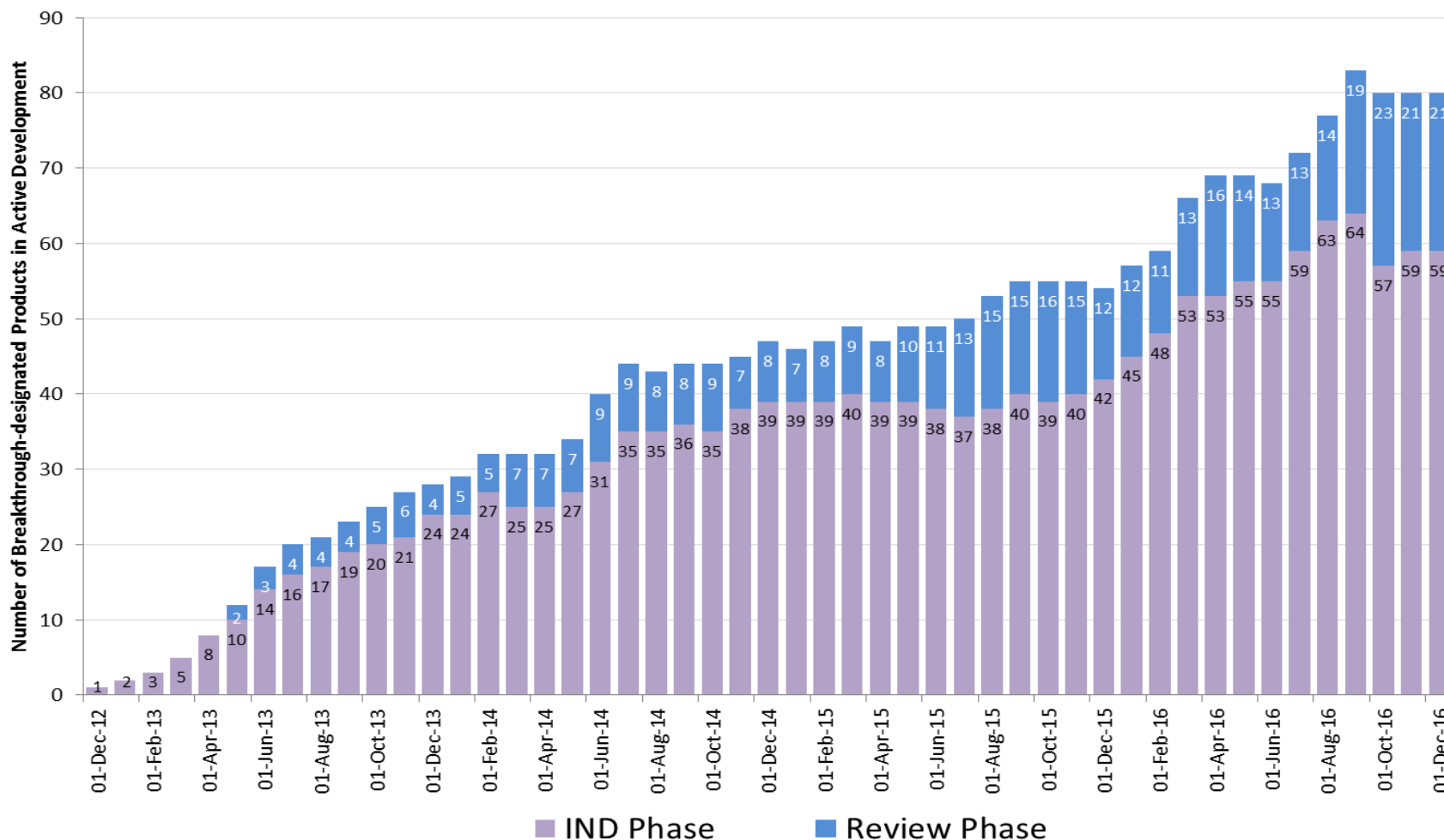
Breakthrough Therapies

- FDASIA program to expedite development and approval of new drugs intended to treat a serious condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies
- FDASIA endorsed and extended FDA's long-standing policy of expediting promising new drugs for serious and life-threatening conditions
- Final guidance "Expedited Programs for Serious Conditions—Drugs and Biologics" issued May 2014

CDER Breakthrough-designated Approvals

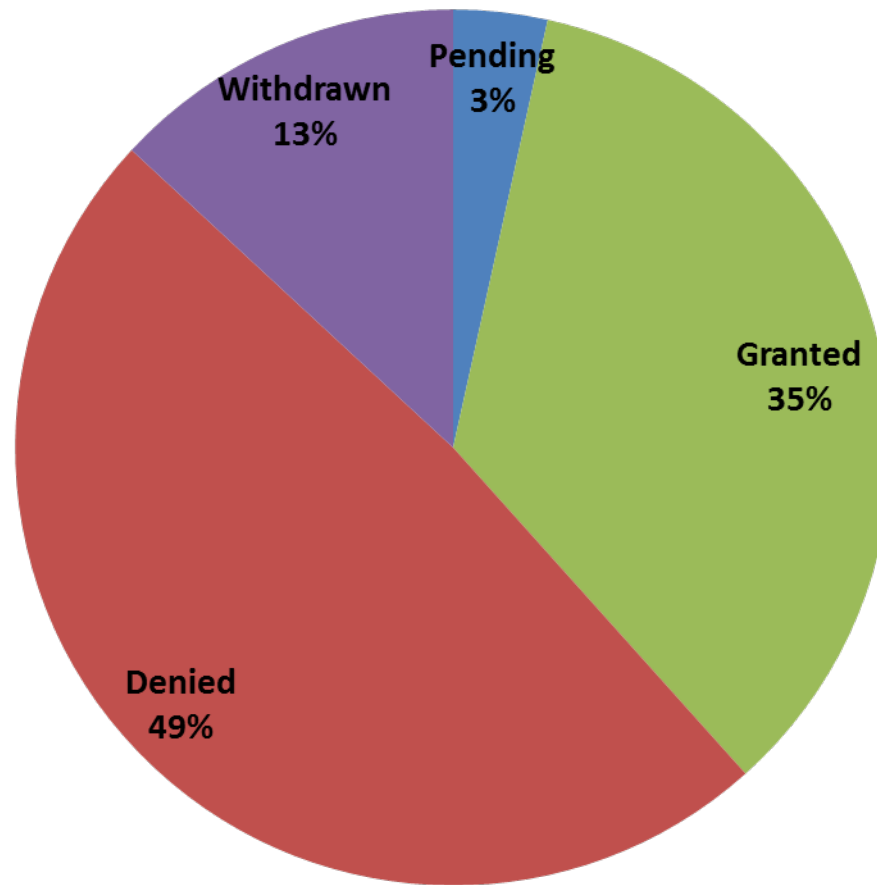


CDER Number of Breakthrough-designated Development Programs Continues to Grow



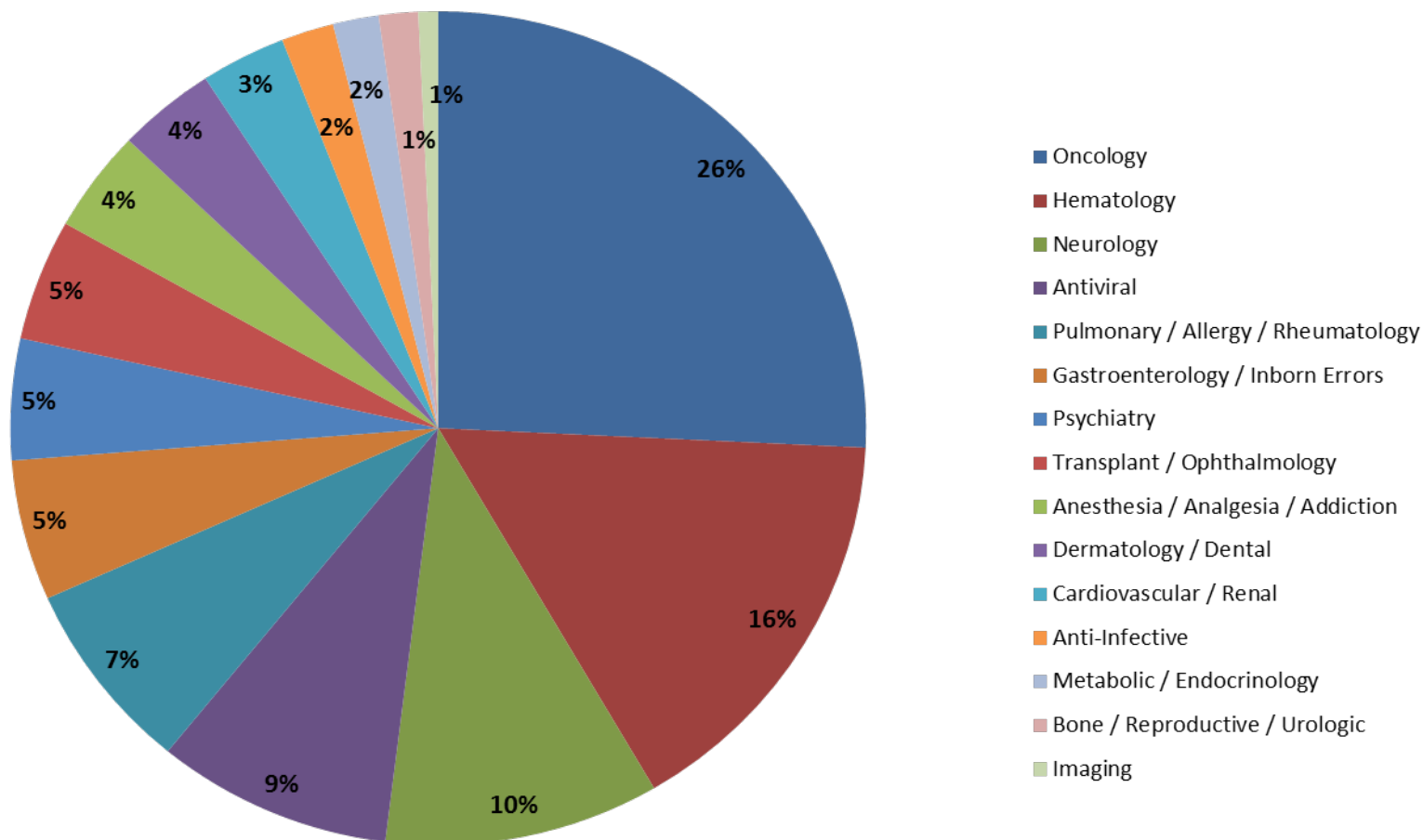
* Data as of 12/1/16. Figures include total # of granted breakthrough designations for drug/indications under active IND development but have not yet received marketing approval or rescission decision.

Current Status of 404 CDER Breakthrough Therapy Requests



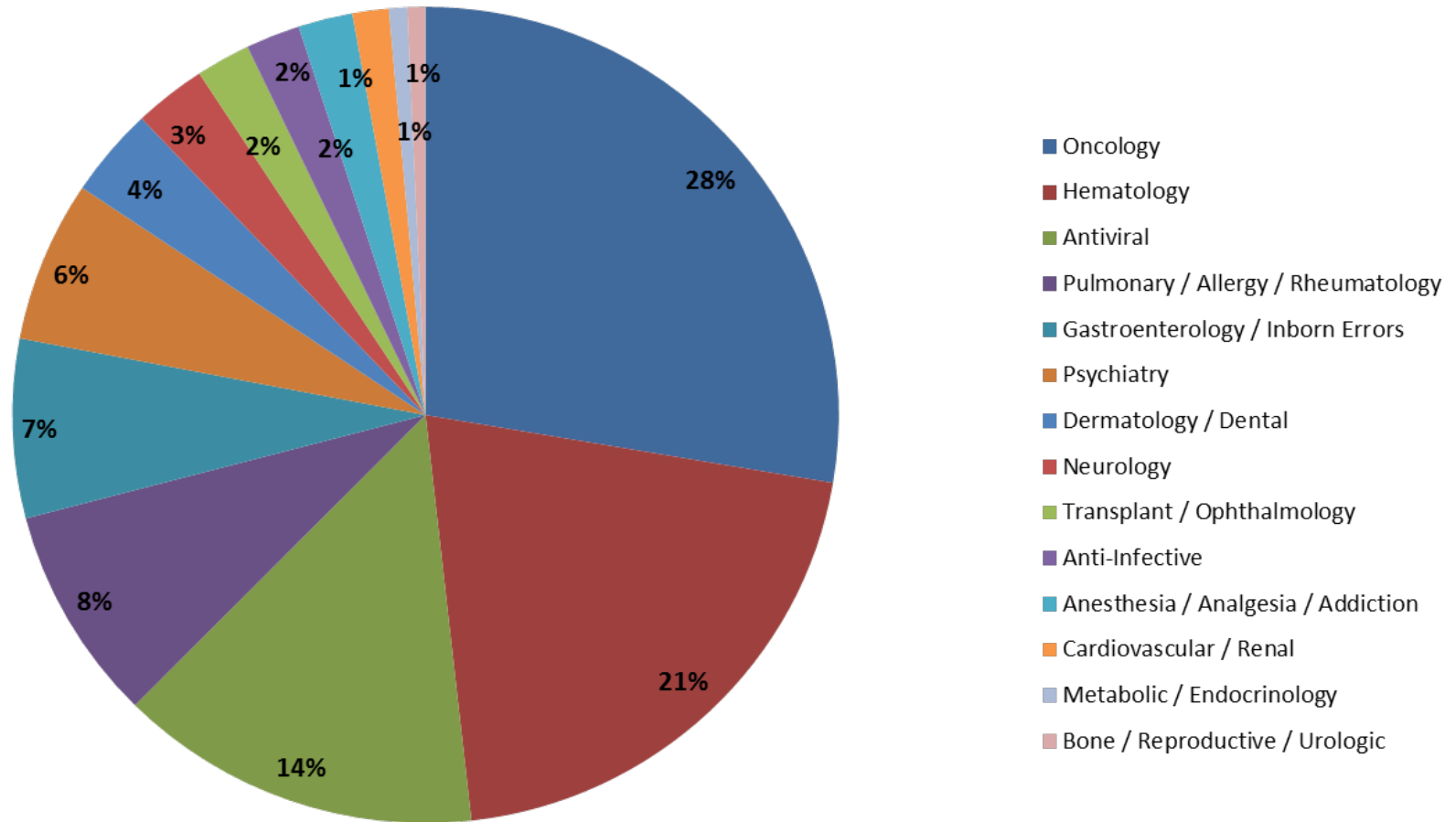
Data as of 11/30/2016

CDER Breakthrough Therapy Requests by Division



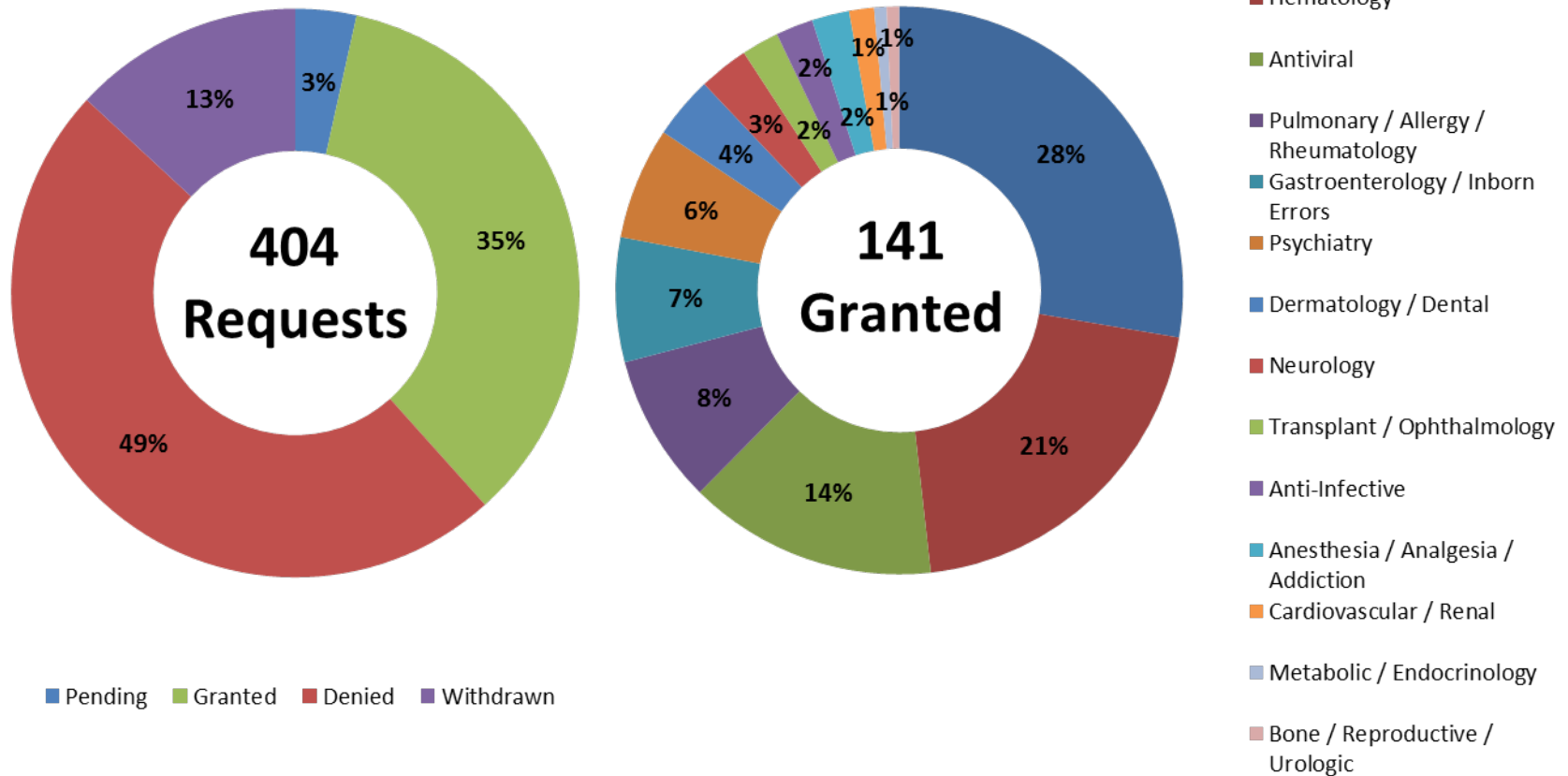
Data as of 11/30/2016

CDER Breakthrough Therapy Requests Granted by Division



Data as of 11/30/2016

CDER Has Granted 141 Breakthrough Therapy Designations Since Inception



Data as of 11/30/2016



Thank You!